UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 10-Q

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended September 30, 2017

or

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from to

Commission File Number: 000-55764

EXICURE, INC.
(Exact name of registrant as specified in its charter)

Delaware	**81-5333008**
(State or other jurisdiction of incorporation or organization)	**(IRS Employer Identification No.)**

8045 Lamon Avenue
Suite 410
Skokie, IL 60077
(Address of principal executive offices)

Registrant's telephone number, including area code (847) 673-1700

Max-1 Acquisition Corporation
2255 Glades Road, Suite 324A
Boca Raton, Florida 33431
(Former name or former address, if changed since last report)

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☐ (Do not check if a smaller reporting company)	Smaller reporting company	☒
		Emerging growth company	☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☒

Indicate by check mark whether the registrant is a shell company (as defined by Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of November 13, 2017, there were 39,300,823 shares of the registrant's common stock, par value $0.0001 per share, outstanding.

EXICURE, INC.

QUARTERLY REPORT ON FORM 10-Q

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains express or implied forward-looking statements that are based on our management's belief and assumptions and on information currently available to our management. All statements other than statements of historical fact contained in this Quarterly Report on Form 10-Q are forward-looking statements. In some cases, you can identify forward-looking statements by terminology such as "may," "could," "will," "would," "should," "expect," "plan,", "anticipate," "believe," "estimate," "intend," "predict," "seek," "contemplate," "project," "continue," "potential," "ongoing" or the negative of these terms or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- the initiation, timing, progress and results of our research and development programs, preclinical studies, clinical trials and Investigational New Drug application ("IND"), Investigational Medicinal Product Dossier, Clinical Trial Application ("CTA"), New Drug Application ("NDA"), or other regulatory submissions;
- our dependence on current and future collaborators for developing, obtaining regulatory approval for and commercializing therapeutic candidates in the collaboration;
- our receipt and timing of any milestone payments or royalties under any current or future research collaboration and license agreements or arrangements;
- our ability to identify and develop therapeutic candidates for treatment of additional disease indications;
- our or a current or future collaborator's ability to obtain and maintain regulatory approval of any of our therapeutic candidates;
- the rate and degree of market acceptance of any approved therapeutic candidates;
- the commercialization of any approved therapeutic candidates;
- our ability to establish and maintain collaborations and retain commercial rights for our therapeutic candidates in the collaborations;
- the implementation of our business model and strategic plans for our business, technologies and therapeutic candidates;
- our estimates of our expenses, ongoing losses, future revenue and capital requirements, including our expectations relating to the use of proceeds from our private placement offering, and our needs for additional financing;
- our ability to obtain additional funds for our operations;
- our ability to obtain and maintain intellectual property protection for our technologies and therapeutic candidates and our ability to operate our business without infringing the intellectual property rights of others;
- our reliance on third parties to conduct our preclinical studies and clinical trials;
- our reliance on third party supply and manufacturing partners to supply the materials and components for, and manufacture, our research and development, preclinical and clinical trial supplies;
- our ability to attract and retain qualified key management and technical personnel;
- our expectations regarding the time during which we will be an emerging growth company under the JOBS Act;
- our financial performance;
- the impact of government regulation and developments relating to our competitors or our industry; and

- other risks and uncertainties, including those listed in Part II, Item 1A—“Risk Factors.”

These statements relate to future events or our future operational or financial performance, and involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by these forward-looking statements. Factors that may cause actual results to differ materially from current expectations include, among other things, those listed in Part II, Item 1A—“Risk Factors” and elsewhere in this Quarterly Report on Form 10-Q.

Any forward-looking statement in this Quarterly Report on Form 10-Q reflects our current view with respect to future events and is subject to these and other risks, uncertainties and assumptions relating to our business, results of operations, industry and future growth. Given these uncertainties, you should not place undue reliance on these forward-looking statements. No forward-looking statement is a guarantee of future performance. You should read this Quarterly Report on Form 10-Q and the documents that we reference in this Quarterly Report on Form 10-Q and have filed with the Securities and Exchange Commission (the "SEC") as exhibits hereto completely and with the understanding that our actual future results may be materially different from any future results expressed or implied by these forward-looking statements. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

This Quarterly Report on Form 10-Q also contains estimates, projections and other information concerning our industry, our business and the markets for certain therapeutics, including data regarding the estimated size of those markets, their projected growth rates and the incidence of certain medical conditions. Information that is based on estimates, forecasts, projections or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances reflected in this information. Unless otherwise expressly stated, we obtained these industry, business, market and other data from reports, research surveys, studies and similar data prepared by third parties, industry, medical and general publications, government data and similar sources. In some cases, we do not expressly refer to the sources from which these data are derived.

As used in this Quarterly Report on Form 10-Q, unless otherwise stated or the context otherwise indicates, references to "Exicure," the "Company," "we," "our," "us," or similar terms refer to Exicure, Inc. and our wholly-owned subsidiary, Exicure Operating Company. Exicure Operating Company holds all material assets and conducts all business activities and operations of the Company.

PART I - FINANCIAL INFORMATION

Item 1. Financial Statements.

EXICURE, INC.

CONSOLIDATED BALANCE SHEETS

(in thousands, except share and per share data)

	September 30, 2017	December 31, 2016
	(unaudited)	
ASSETS		
Current assets:		
Cash and cash equivalents	$ 22,936	$ 19,623
Unbilled revenue receivable	201	—
Receivable from related party (Note 12)	13	15
Prepaid expenses and other assets	1,130	403
Total current assets	24,280	20,041
Property and equipment, net (Note 4)	1,059	503
Other noncurrent assets	32	32
Total assets	$ 25,371	$ 20,576
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Current portion of long-term debt (Note 5)	$ 2,551	$ 1,213
Accounts payable	2,379	509
Accrued expenses and other current liabilities (Note 4)	2,496	2,160
Current portion of deferred revenue (Note 3)	3,103	8,276
Total current liabilities	10,529	12,158
Long-term debt, net (Note 4)	2,670	4,454
Preferred stock warrant liability (Note 10)	—	201
Common stock warrant liability (Note 10)	211	—
Deferred revenue, net of current portion (Note 3)	—	1,034
Other noncurrent liabilities	279	281
Total liabilities	$ 13,689	$ 18,128
Stockholders' equity (Note 6):		
Non-redeemable preferred stock		
Series C: $0.00001 par value per share; no shares authorized, issued, and outstanding, September 30, 2017; 16,100,000 shares authorized; 11,239,359 shares issued and outstanding, December 31, 2016	—	33,483
Series B-2: $0.00001 par value per share; no shares authorized, issued, and outstanding, September 30, 2017; 1,403,984 shares authorized, issued and outstanding, December 31, 2016	—	3,641
Series B-1: $0.00001 par value per share; no shares authorized, issued, and outstanding, September 30, 2017; 2,451,560 shares authorized, issued and outstanding, December 31, 2016	—	5,371
Series A: $0.00001 par value per share; no shares authorized, issued, and outstanding, September 30, 2017; 11,381,640 shares authorized, issued and outstanding, December 31, 2016	—	135
Common stock, $0.0001 par value per share; 200,000,000 shares authorized, 35,513,987 issued and outstanding, September 30, 2017; 30,782,380 shares authorized, 131,644 issued and outstanding, December 31, 2016	4	—
Additional paid-in capital	43,219	(17,578)
Accumulated deficit	(31,541)	(22,604)
Total stockholders' equity	11,682	2,448
Total liabilities and stockholders' equity	$ 25,371	$ 20,576

See Accompanying Notes to Unaudited Consolidated Financial Statements.

EXICURE, INC.

UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

(in thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
Revenue:				
Collaboration revenue	$ 2,497	$ —	$ 7,624	$ —
Grant income	—	—	—	346
Total revenue	2,497	—	7,624	346
Operating expenses:				
Research and development expense	3,502	2,413	11,279	8,329
General and administrative expense	1,270	849	4,806	2,736
Total operating expenses	4,772	3,262	16,085	11,065
Operating loss	(2,275)	(3,262)	(8,461)	(10,719)
Other income (expense), net:				
Interest expense	(201)	(208)	(616)	(516)
Other income (loss), net	163	(56)	140	(64)
Total other income (loss), net	(38)	(264)	(476)	(580)
Net loss	$ (2,313)	$ (3,526)	$ (8,937)	$ (11,299)
Basic and diluted loss per common share	$ (1.34)	$ (36.09)	$ (48.73)	$ (103.15)
Basic and diluted weighted-average common shares outstanding	1,725,906	97,691	183,395	109,539

See Accompanying Notes to Unaudited Consolidated Financial Statements.

EXICURE, INC.

UNAUDITED CONSOLIDATED STATEMENT OF CHANGES IN STOCKHOLDERS' EQUITY

(in thousands, except shares)

	Non-Redeemable Preferred Stock								Common Stock				
	Series C		Series B-2		Series B-1		Series A						
	Shares	$	Shares	$	Shares	$	Shares	$	Shares	$	Additional Paid-in-Capital	Accumulated Deficit	Total Stockholders' Equity
Balance at December 31, 2016	**11,239,359**	**$33,483**	**1,403,984**	**$3,641**	**2,451,560**	**$5,371**	**11,381,640**	**$135**	**131,644**	**$—**	**$(17,578)**	**$(22,604)**	**$ 2,448**
Exercise of options	—	—	—	—	—	—	—	—	58,440	—	43	—	43
Equity-based compensation	—	—	—	—	—	—	—	—	—	—	1,104	—	1,104
Share conversion in connection with the Merger	(11,239,359)	(33,483)	(1,403,984)	(3,641)	(2,451,560)	(5,371)	(11,381,640)	(135)	28,556,543	3	42,596	—	(31)
Issuance of common stock, net	—	—	—	—	—	—	—	—	6,767,360	1	17,054	—	17,055
Net loss	—	—	—	—	—	—	—	—	—	—	—	(8,937)	(8,937)
Balance at September 30, 2017	**—**	**$ —**	**—**	**$ —**	**—**	**$ —**	**—**	**$ —**	**35,513,987**	**$ 4**	**$ 43,219**	**$(31,541)**	**$ 11,682**

See Accompanying Notes to Unaudited Consolidated Financial Statements.

EXICURE, INC.

UNAUDITED CONSOLIDATED STATEMENTS OF CASH FLOWS

(in thousands)

	Nine Months Ended September 30,	
	2017	**2016**
Cash flows from operating activities:		
Net loss	$ (8,937)	$ (11,299)
Adjustments to reconcile net loss to cash used in operating activities:		
Depreciation and amortization	159	132
Equity-based compensation	1,104	507
Amortization of long-term debt issuance costs and fees	150	137
Change in fair value of warrant liabilities	(201)	67
Changes in operating assets and liabilities:		
Unbilled revenue receivable and accounts receivable	(201)	237
Receivable from related party	2	49
Prepaid expenses and other current assets	(727)	(151)
Accounts payable	1,361	(359)
Accrued expenses and other current liabilities	(948)	2
Deferred revenue	(6,207)	—
Other noncurrent liabilities	(2)	1
Net cash used in operating activities	(14,447)	(10,677)
Cash flows from investing activities:		
Capital expenditures	(726)	(315)
Net cash used in investing activities	(726)	(315)
Cash flows from financing activities:		
Proceeds from common stock offering	20,302	—
Proceeds from preferred stock offering	—	450
Proceeds from long-term borrowing	—	6,000
Proceeds from exercise of common stock options	43	26
Repayment of long-term debt	(595)	—
Payment of long-term debt fees and issuance costs	—	(141)
Payment of common stock financing costs	(1,264)	—
Payment of preferred stock financing costs	—	(6)
Net cash provided by financing activities	18,486	6,329
Net increase (decrease) in cash and cash equivalents	3,313	(4,663)
Cash and cash equivalents - beginning of period	19,623	18,731
Cash and cash equivalents - end of period	$ 22,936	$ 14,068
Supplemental disclosure of cash flow information		
Non-cash financing activities:		
Common stock issuance costs (accounts payable and accrued expenses)	$ 1,773	$ —
Issuance of common stock warrants	211	—
Issuance of preferred stock warrants	—	134
Debt issuance costs (accounts payable)	—	231

See Accompanying Notes to Unaudited Consolidated Financial Statements.

1. Description of Business and Basis of Presentation

Description of Business

Exicure, Inc. ("Parent") is a clinical-stage biotechnology company developing gene regulatory and immuno-oncology therapeutics based on the Company's proprietary Spherical Nucleic Acid ("SNA") technology. We believe the design of the Company's SNAs gives rise to chemical and biological properties that may provide advantages over other nucleic acid therapeutics and enable therapeutic activity outside of the liver. The Company intends to build a leading nucleic acid therapeutics company focused on the discovery and development of therapeutics based on the Company's proprietary SNA technology, either on its own or in collaboration with pharmaceutical partners.

Throughout this Quarterly Report on Form 10-Q, the terms "the Company" and "Exicure" refer to Parent and its 100% owned subsidiary, Exicure Operating Company. Exicure Operating Company holds all material assets, and conducts all business activities and operations, of the Company.

The Merger

On September 26, 2017, pursuant to the merger agreement, Max-1 Acquisition Sub, Inc., a wholly-owned subsidiary of Max-1 Acquisition Corporation ("Max-1"), merged with and into Exicure, Inc. (formerly AuraSense Therapeutics, LLC), a privately-held Delaware corporation referred to herein as Exicure OpCo., with Exicure OpCo remaining as the surviving entity and a wholly-owned operating subsidiary of Max-1 Acquisition Corporation (the "Merger"). The Merger was effective as of September 26, 2017 (the "Effective Time"), upon the filing of a Certificate of Merger with the Secretary of State of the State of Delaware.

At the Effective Time, the legal existence of Max-1 Acquisition Sub, Inc. ceased. At the Effective Time, each share of Exicure OpCo common and preferred stock (other than shares of Exicure OpCo's Series C preferred stock) issued and outstanding immediately prior to the closing of the Merger was converted into 0.49649 shares of Max-1 common stock, and each share of Exicure OpCo's Series C preferred stock issued and outstanding immediately prior to the closing of the Merger was converted into 0.7666652 shares of Max-1 common stock. As a result, an aggregate of 26,666,627 shares of Max-1 common stock were issued to the holders of Exicure OpCo's capital stock, which is incremental to the 2,080,000 shares of Max-1 common stock that were outstanding immediately prior to the Merger. In addition, pursuant to the Merger Agreement, options to purchase 7,414,115 shares of Exicure OpCo common stock issued and outstanding immediately prior to the closing of the Merger were assumed by Max-1 and converted into options to purchase 3,680,997 shares of Max-1 common stock. After the filing of the Certificate of Merger with the Secretary of State of the State of Delaware, Max-1 changed its name to Exicure, Inc.

The Merger is considered a "reverse merger," whereby Exicure OpCo is considered the accounting acquirer in the Merger. Exicure OpCo was determined to be the accounting acquirer based on the terms of the Merger and other factors including: (i) legacy Exicure OpCo shareholders own approximately 94% of the combined company on a fully diluted basis immediately following the closing of the Merger, (ii) legacy Exicure OpCo directors will hold all six board seats of the combined company, and (iii) legacy Exicure OpCo management will hold all positions in management of the combined company. The transaction is accounted for as an asset acquisition rather than a business combination because as of the acquisition date, Max-1 does not meet the definition of a business as defined by accounting principles generally accepted in the United States of America ("GAAP"). Consequently, the assets, liabilities and operations that are reflected in Exicure's historical financial statements prior to the Merger will be those of Exicure OpCo, and the consolidated financial statements after completion of the Merger will include the assets, liabilities and results of operations of Exicure OpCo up to the day prior to the closing of the Merger and the assets, liabilities and results of operations of the combined company from and after the closing date of the Merger. The assets and liabilities of Max-1 included in the accompanying consolidated financial statements are recorded at the historical cost basis of Max-1.

In this Quarterly Report on Form 10-Q, unless otherwise indicated, all share and per share figures are retrospectively adjusted to reflect the conversion of each share of Exicure OpCo common and preferred stock (other than shares of Exicure OpCo's Series C preferred stock), preferred stock warrant liability, and common stock options issued and outstanding immediately prior to the closing of the Merger into 0.49649 shares of the Company's common stock, and each share of Exicure OpCo's Series C preferred stock issued and outstanding immediately prior to the closing of the Merger into 0.7666652 shares of the Company's common stock.

Capitalization Prior to the Merger

AuraSense Therapeutics, LLC was formed on June 13, 2011 as a wholly owned subsidiary of AuraSense, LLC, but did not conduct substantive business until December 12, 2011, which is considered the inception date. On December 12, 2011, AuraSense, LLC contributed the assets and liabilities comprising the business of the Company to the Company through a Bill of Sale and Assumption Agreement. Pursuant to this agreement AuraSense, LLC received 11,381,611 Class A Units of the Company.

The assets and liabilities contributed by AuraSense, LLC were transferred at their historical cost and consisted of an unbilled revenue receivable of $143, scientific equipment of $309 and a liability of $317 for accrued legal expenses related to patent protection. The net book value of Parent’s contribution at inception was $135.

Also on December 12, 2011, the Company and AuraSense, LLC entered into a Partial Assignment of License Agreement whereby certain license rights held by AuraSense, LLC pursuant to a License Agreement with Northwestern University were assigned to the Company. Under the terms of the License Agreement and the Partial Assignment of License Agreement, Northwestern University received 1.0% of the Class A units received by AuraSense, LLC in the formation transaction, which amounted to 113,816 units.

On July 9, 2015, AuraSense Therapeutics, LLC was converted into AuraSense Therapeutics, Inc., a Delaware corporation, and on the same date changed its name to Exicure, Inc., which actions together are referred to in these Notes to Unaudited Consolidated Financial Statements as the corporate conversion. In connection with the corporate conversion, each common unit, Class A unit, Class B-1 unit, Class B-2 unit and Class C unit of AuraSense Therapeutics, LLC issued and outstanding immediately prior to the effectiveness of the corporate conversion was converted into one share of common stock, Series A preferred stock, Series B-1 preferred stock, Series B-2 preferred stock and Series C preferred stock of Exicure OpCo, respectively. No preferred stock was provided in consideration for fractional membership units. Each outstanding option to purchase one common unit of AuraSense Therapeutics, LLC was converted into an option to purchase one share of common stock of Exicure OpCo. In connection with the corporate conversion, the accumulated deficit of AuraSense Therapeutics, LLC of $18,837 was reclassified to Additional paid in capital.

Refer to Note 6, *Stockholders' Equity,* for more information on capital stock transactions.

Basis of Presentation

The accompanying consolidated financial statements as of September 30, 2017 and December 31, 2016, and for the three and nine months ended September 30, 2017 and 2016, have been presented in conformity with GAAP.

Principles of consolidation

The accompanying consolidated financial statements include the accounts of Parent and its 100% owned subsidiary, Exicure Operating Company. All intercompany transactions and accounts are eliminated in consolidation.

Liquidity risk

As of September 30, 2017, the Company has generated an accumulated deficit of $50,378 since inception and expects to incur significant expenses and negative cash flows for the foreseeable future. Based on the Company’s current operating plans, it believes that existing working capital at September 30, 2017 when combined with the aggregate cash raised on October 27, 2017 and November 2, 2017 of approximately $11,211 for the sale of common

stock (see Note 13, *Subsequent Events*), but excluding any potential proceeds from option exercises, is sufficient to fund its current operating plans into 2019. Management believes that it will be able to obtain additional working capital through equity financings, partnerships and licensing, or other arrangements, to fund operations. However, there can be no assurance that such additional financing will be available and, if available, can be obtained on terms acceptable to the Company. If the Company is unable to obtain such additional financing, the Company will need to reevaluate future operating plans.

Unaudited interim financial information

The accompanying interim consolidated balance sheet as of September 30, 2017, the interim consolidated statements of operations for the three and nine months ended September 30, 2017 and 2016, the interim consolidated statement of changes in stockholders' equity for the nine months ended September 30, 2017, and the interim consolidated statements of cash flows for the nine months ended September 30, 2017 and 2016, are unaudited. The interim unaudited consolidated financial statements have been prepared on the same basis as the annual audited financial statements; and in the opinion of management, reflect all adjustments, which include only normal recurring adjustments necessary for the fair statement of the Company's financial position as of September 30, 2017, the results of its operations for the three and nine months ended September 30, 2017 and 2016, and the results of its cash flows for the nine months ended September 30, 2017 and 2016. The financial data and other information disclosed in these notes related to the three and nine months ended September 30, 2017 and 2016 are unaudited. The results for the three and nine months ended September 30, 2017 are not necessarily indicative of results to be expected for the year ending December 31, 2017, or any other interim periods, or any future year or period.

Use of estimates

The preparation of the financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of revenues and expenses during the reporting period. Management bases its estimates on certain assumptions which it believes are reasonable in the circumstance and while actual results could differ from those estimates, management does not believe that any change in those assumptions in the near term would have a significant effect on the Company's financial position, results of operations or cash flows. Actual results in future periods could differ from those estimates.

2. Significant Accounting Policies

Cash and cash equivalents

The Company considers all highly liquid investments with original maturities of three months or less to be cash equivalents.

Accounts receivable and unbilled revenue receivable

Accounts receivable and unbilled revenue receivable consist of reimbursement for research and development activities in connection with the research collaboration, license, and option agreement with Purdue Pharma L.P. ("Purdue") and, in 2016, grant proceeds for services under government grant agreements. The Company's management believes these receivables are fully collectible.

Fair value of financial instruments

The carrying amounts of financial instruments, which include cash and cash equivalents and accounts payable, approximate their respective fair values due to the relatively short-term nature of these instruments. Management believes that the Company's long-term debt bears interest at the prevailing market rate for instruments with similar characteristics and, accordingly, the carrying value of long-term debt also approximates their fair value.

Concentrations of credit risk and other risks and uncertainties

Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash and cash equivalents. As of September 30, 2017 and December 31, 2016, the Company had cash and cash equivalents of $22,936 and $19,623, respectively. The cash balances at each respective period were maintained at two institutions. These deposits exceed federally insured limits.

The Company is currently not profitable and no assurance can be provided that it will ever be profitable. The Company's research and development activities have required significant investment since inception and operations are expected to continue to require cash investment in excess of its revenues. See also Note 1, *Description of Business and Basis of Presentation—Liquidity Risk*, for more information.

The Company is subject to risks common in therapeutic development including, but not limited to, therapeutic candidates that appear promising in the early phases of development often fail because they prove to be inefficacious or unsafe, clinical trial results are unsuccessful, regulatory bodies may not approve the therapeutic or the therapeutic may not be economical in production or distribution. The Company is also subject to risks common to biotechnology firms including, but not limited to new and disruptive technological innovations, dependence on key personnel, protection of proprietary technology, the validity of and continued access to its owned and licensed intellectual property, limitations on the supply of critical materials, compliance with governmental regulations and market acceptance.

Property and equipment

Property and equipment are recorded at cost and depreciated using the straight-line method over the estimated useful lives of the various classes of property and equipment, which range from three to seven years. Leasehold improvements are amortized using the straight-line method over the shorter of the remaining terms of the respective leases or the estimated lives of the assets.

Property and equipment are reviewed for impairment whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. No impairment losses were recorded from inception in December 2011 through September 30, 2017.

Common stock warrant liability

Freestanding warrants related to shares that are redeemable, contingently redeemable, or for purchases of common stock that are not indexed to the Company's own stock are classified as a liability on the Company's balance sheet. The common stock warrants are recorded at fair value, estimated using the Black-Scholes option-pricing model, and marked to market at each balance sheet date with changes in the fair value of the liability recorded in other income (expense), net in the statements of operations.

Revenue recognition

The Company recognizes revenue when the following criteria have been met: (1) persuasive evidence of an arrangement exists; (2) delivery has occurred or services have been rendered and risk of loss has passed; (3) the fee is fixed or determinable; and (4) collectability is reasonably assured. When payments are received in advance of recognizing revenue, the Company includes the amount in deferred revenue on the balance sheet. Amounts deferred that are not anticipated to be recognized as revenue within a year of the balance sheet date are classified as noncurrent liabilities.

The Company has generated all of its revenue to date through its research collaboration, license, and option agreement with Purdue Pharma L.P. or as a primary contractor or as a subcontractor on government grants. The Company has not generated any commercial product revenue. Historically, the Company's research collaborations and grants have been either as a direct contractor or as a sub-awardee on contracts funded by various governmental agencies.

In arrangements involving the delivery of more than one element, each required deliverable is evaluated to determine whether it qualifies as a separate unit of accounting. The determination is based on whether the deliverable has "standalone value" to the customer. If a deliverable does not qualify as a separate unit of accounting, it is combined with the other applicable undelivered item(s) within the arrangement and these combined deliverables are treated as a single unit of accounting.

The arrangement's consideration that is fixed or determinable is allocated to each separate unit of accounting based on the relative selling price methodology in accordance with the selling price hierarchy, which includes vendor-specific objective evidence ("VSOE") of selling price, if available, or third-party evidence of selling price if VSOE is not available, or the best estimate of selling price, if neither VSOE nor third-party evidence is available.

Payments or reimbursements for the Company's research and development efforts for the arrangements where such efforts are considered as deliverables are recognized as the services are performed and are presented on a gross basis. When upfront payments are received and if there is no discernible pattern of performance and/or objectively measurable performance measures do not exist, the Company recognizes revenue ratably over the associated period of performance.

The Company's grant contracts have typically been cost or cost-plus-fee contracts. Revenues on these contracts are recognized as costs are incurred, generally based on allowable costs incurred during the period, plus any recognizable earned fee. The Company considers fixed fees under cost and cost-plus-fee contracts to be earned in proportion to the allowable costs incurred in performance of the contract. The Company has determined that it is the principal for each of its grants with governmental agencies since it maintains primary responsibility for research efforts in connection with these grants. Therefore, it recognizes revenue related to these grants with governmental agencies on a gross basis.

The Company has entered into certain grant contracts containing milestone payments. The Company recognizes revenue from milestone payments when earned, provided that the milestone event was substantive, its achievability was not reasonably assured at the inception of the agreement, the Company has no further performance obligations relating to the event and collectability is reasonably assured. If these criteria are not met, the Company recognizes milestone payments ratably over the remaining period of its performance obligations under the grant contract. For a milestone to be considered substantive, the payment associated with its achievement must have all of the following characteristics: (1) relate solely to past performance; (2) be reasonable, relative to all of the deliverables and payment terms within the arrangement; and (3) be commensurate with either the Company's efforts required to achieve the milestone or the enhanced value of the delivered items(s) as a result of the milestone achievement.

As of September 30, 2017 and December 31, 2016, and for the three and nine months ended September 30, 2017 and 2016, the Company was not participating in any grant research activities involving milestone payments.

Equity-based compensation

The Company measures the cost of common stock option awards at fair value and records the cost of the awards, net of estimated forfeitures, on a straight-line basis over the requisite service period. The Company measures fair value for all common stock options using the Black-Scholes option-pricing model. For all common stock option awards to employees, the fair value measurement date is the date of grant and the requisite service period is the period over which the employee is required to provide service in exchange for the common stock option awards, which is generally the vesting period. For all common stock option awards to nonemployees, the

Company remeasures fair value at each financial statement reporting date and recognizes compensation expense as services are rendered, generally on a straight-line basis.

Segments and geographic information

The Company has determined it has one operating segment. Disaggregating the Company's operations is impracticable because the Company's research and development activities and its assets overlap and management reviews its business as a single operating segment. Thus, discrete financial information is not available by more than one operating segment. All long-lived assets of the Company are located in the United States.

Deferred rent

Deferred rent consists of rent escalation payment terms, tenant improvement allowances and other incentives received from the landlord related to the Company's operating lease and is presented in "Other noncurrent assets" in the accompanying balance sheet. Rent escalation represents the difference between actual operating lease payments due and straight-line rent expense, which is recorded by the Company over the term of the lease. Tenant improvement allowances and other incentives are recorded as deferred rent and amortized as a reduction of periodic rent expense, over the term of the applicable lease.

Research and development expense

Research and development expense includes wages, benefits, research materials, external services, legal fees related to patent protection, overhead and other expenses directly related to research and development operations. Research and development costs are expensed as incurred in accordance with ASC 730, Research and Development.

Income taxes

From inception through July 9, 2015, the Company was a Delaware LLC for federal and state tax purposes and, therefore, all items of income or loss through July 9, 2015 flowed through to the members of AuraSense Therapeutics, LLC. Effective July 9, 2015, the Company converted from an LLC to a C corporation for federal and state income tax purposes. Accordingly, prior to the conversion to a C corporation, the Company did not record deferred tax assets or liabilities or have any net operating loss carryforwards. The Company recognizes deferred tax assets and liabilities for temporary differences between the financial reporting basis and the tax basis of its assets and liabilities and the expected benefits of net operating loss carryforwards. The impact of changes in tax rates and laws on deferred taxes, if any, is applied during the years in which temporary differences are expected to be settled and is reflected in the financial statements in the period of enactment. The measurement of deferred tax assets is reduced, if necessary, if, based on weight of the evidence, it is more likely than not that some, or all, of the deferred tax assets will not be realized. At September 30, 2017 and December 31, 2016, the Company established a full valuation allowance against its deferred tax assets.

Recent accounting pronouncements

In May 2014, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") 2014-09 (ASC 606), Revenue from Contracts with Customers. This ASU, as amended by ASU 2015-14, affects any entity that either enters into contracts with customers to transfer goods and services or enters into contracts for the transfer of nonfinancial assets. ASU 2014-09 will replace most existing revenue recognition guidance in GAAP when it becomes effective. The standard's core principle is that a company will recognize revenue when it transfers promised goods or services to customers in an amount that reflects the consideration to which the company expects to be entitled in exchange for those goods or services. In doing so, companies will need to use more judgment and make more estimates than under the currently effective guidance. These may include identifying performance obligations in the contract, estimating the amount of variable consideration to include in the transaction price and allocating the transaction price to each separate performance obligation. ASU 2014-09 is effective for Exicure in the first quarter of 2018 and early adoption is permitted beginning in the first quarter of 2017. The Company is currently evaluating the impact of this guidance on its financial statements.

In February 2016, FASB issued ASU 2016-02, Leases (Topic 842), which requires lessees to recognize right-of-use assets and lease liabilities on the balance sheet. ASU 2016-02 is to be applied using a modified retrospective approach at the beginning of the earliest comparative period in the financial statements. ASU 2016-02 will be effective for the Company beginning in the first quarter of 2019. Early adoption is permitted. The Company is currently evaluating the impact of adopting this standard on its financial statements.

In March 2016, the FASB issued ASU 2016-09, Improvements to Employee Share-Based Payment Accounting. ASU 2016-09 changes several aspects of the accounting for share-based payment transactions, including the income tax consequences, classification of awards as either equity or liabilities, and classification on the statement of cash flows. ASU 2016-09 requires all excess tax benefits and tax deficiencies to be recognized as income tax expense or benefit in the income statement and treated as discrete items in the reporting period. Further, excess tax benefits are required to be classified along with other income tax cash flows as an operating activity. The guidance was effective for the Company in the first quarter of 2017. Adoption of this guidance did not have a significant impact to the Company's financial statements.

In August 2016, the FASB issued ASU 2016-15, Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments. ASU 2016-15 addresses the classification of certain specific cash flow issues including debt prepayment or extinguishment costs, settlement of certain debt instruments, contingent consideration payments made after a business combination, proceeds from the settlement of certain insurance claims and distributions received from equity method investees. ASU 2016-15 is effective for the Company in the first quarter of 2018 and early adoption is permitted. An entity that elects early adoption must adopt all of the amendments in the same period. The Company is currently evaluating the impact of this guidance on its statement of cash flows.

In May 2017, the FASB issued ASU 2017-09, Compensation - Stock Compensation (Topic 718): Scope of Modification Accounting. ASU 2017-09 clarifies when changes to the terms or conditions of a share-based payment award must be accounted for as modifications. Under the new guidance, modification accounting is required only if the fair value, the vesting conditions, or the classification of the award changes as a result of the change in terms or conditions. ASU 2017-09 will be applied prospectively to awards modified on or after the adoption date. ASU 2017-09 is effective for the Company for fiscal years beginning after December 15, 2017, and interim periods within those fiscal years. Early adoption is permitted. The Company is currently evaluating the impact of this guidance on its financial statements.

3. Purdue Collaboration

On December 2, 2016, the Company entered into a research collaboration, option and license agreement with Purdue and referred to herein as the "Purdue Collaboration." Purdue has the option to obtain from us the full worldwide development and commercial rights to AST-005 (the Company's lead therapeutic candidate that targets tumor necrosis factor), an option to obtain three additional collaboration targets and a further option to obtain from us the full worldwide development and commercial rights to any therapeutic candidates developed targeting the three additional collaboration targets. Additionally, Purdue has rights of first offer to some potential collaboration targets. These rights of first offer are subject to limitations in time and scope. In connection with the Purdue Collaboration, the Company received a non-refundable development fee of $10,000. In addition, the Company is eligible to receive up to $776,500 upon successful completion of certain research, regulatory and commercial sales milestones. The research milestones are payable upon target identification and IND-enabling pre-clinical development, per program, with an aggregate total of up to $16,500. The regulatory milestones are payable upon the initiation or completion of clinical trials, and regulatory approval in the United States and outside the United States, per program, with an aggregate total of up to $410,000. The commercial sales milestones are payable upon achievement of specified aggregate product sales thresholds and total up to $350,000. In the event a therapeutic candidate subject to the collaboration results in commercial sales, the Company is eligible to receive royalties ranging from the low single digits to a maximum of 10% on future net sales of such commercialized therapeutic candidates. Additionally, Purdue had an obligation to invest in a qualified equity financing of the Company if such financing was completed before June 2, 2017. The Company did not complete such qualified equity financing before June 2, 2017.

In accordance with ASC 605-25, the Company identified the following deliverables at the inception of the Purdue Collaboration agreement: (1) exclusive rights to the TNF-α target, (2) the obligation to participate in a joint research committee, (3) the provision of research and development activities based on a prescribed full-time employee rate per year, (4) a non-voting board of director observer role, (5) Purdue's right to participate in a future qualified equity financing of the Company if such financing occurs prior to June 2, 2017 or the pricing of the initial public offering of shares of the Company's common stock, (6) the option for an exclusive development and commercialization license to AST-005 or a TNF-α development candidate other than AST-005, (7) the option to select and develop three additional collaboration targets, and (8) the option for an exclusive development and commercialization license to any developed therapeutic candidate targeting the three additional collaboration targets. The Company determined that deliverables (2), (4), (5), and (6) do not have stand-alone value to Purdue, and accordingly, deliverables (2), (4), (5) and (6) were combined with deliverables (1) and (3) as a single unit of accounting. The Company concluded that, at the inception of the agreement, deliverables (7) and (8) are substantive options and do not contain a significant or incremental discount; as a result, no portion of the upfront $10,000 is allocated to deliverables (7) and (8).

The upfront payment of $10,000 was allocated to the single unit of accounting consisting of deliverables (1), (2), (3), (4), (5), and (6) above and was recorded as deferred revenue and is being recognized on a ratable basis over the estimated performance period of the relevant research and development activities of 14.5 months.

The Purdue Collaboration agreement includes contingent payments related to specified research, development and regulatory milestones and sales-based milestones. Each contingent and milestone payment is evaluated to determine whether it is substantive and at risk to both parties. The Company recognizes any payment that is contingent upon the achievement of a substantive milestone entirely in the period in which the milestone is achieved. Any payments that are contingent upon achievement of a non-substantive milestone are recognized as revenue prospectively, when such payments become due and collectible, over the remaining expected performance period under the arrangement, which is generally the remaining period over which the research and development services are expected to be provided. To date, the Company has not recognized any contingent payments in connection with the Purdue Collaboration as revenue.

During the three months ended September 30, 2017, the Company recognized collaboration revenue of $2,497, which included $428 of research and development activities that will be reimbursed by Purdue and is presented on a gross basis in the accompanying statement of operations. During the nine months ended September 30, 2017, the Company recognized collaboration revenue of $7,624, which included $1,417 of research and development activities that will be reimbursed by Purdue and is presented on a gross basis in the accompanying statement of operations. As of September 30, 2017 and December 31, 2016, deferred revenue relating to the Purdue Collaboration was $3,103 and $9,310, respectively, of which $3,103 and $8,276, respectively, is classified as current portion of deferred revenue in the accompanying balance sheet.

(in thousands, except unit, share, per unit, and per share data)

4. Supplemental Balance Sheet Information

Property and equipment, net

	September 30, 2017	December 31, 2016
Scientific equipment	$ 1,586	$ 993
Leasehold improvements	192	57
Furniture and fixtures	31	27
Computers and software	26	26
Construction in process	—	17
Property and equipment, gross	1,835	1,120
Less: accumulated depreciation	(776)	(617)
Property and equipment, net	$ 1,059	$ 503

Depreciation and amortization expense was $70 and $46 for the three months ended September 30, 2017 and 2016, respectively, and $159 and $132 for the nine months ended September 30, 2017 and 2016, respectively.

Accrued expenses and other current liabilities

	September 30, 2017	December 31, 2016
Accrued Northwestern University License Agreements fee (Note 11)	$ —	$ 1,500
Accrued legal expenses	1,004	123
Accrued payroll-related expenses	513	423
Other accrued expenses	979	114
Accrued expenses and other current liabilities	$ 2,496	$ 2,160

5. Debt

On February 17, 2016, the Company closed a $10,000 loan facility, with an initial advance against this loan facility of $6,000, with Hercules Technology Growth Capital ("Hercules"). The loan bears a floating interest rate equal to the greater of either (i) 9.95% or (ii) the sum of 9.95% plus the United States prime rate minus 3.50%. Total proceeds net of fees and issuance costs were $5,839. Fees and issuance costs of $161, as well as fees of $231 that are payable to the lender at maturity, are recorded as a reduction in the carrying amount of long-term debt on our balance sheet and will be amortized to interest expense through the maturity date of September 1, 2019 using the effective interest method. Interest amounts were payable monthly beginning on March 1, 2016 through the maturity date of September 1, 2019. Initially, principal amounts were payable monthly beginning on April 1, 2017 through the maturity date. In 2016, the Company met certain terms in the loan agreement so that principal amounts became payable monthly beginning on July 1, 2017. The loan is collateralized by a security interest in all tangible assets. In addition, the Company is subject to certain financial reporting requirements and certain negative covenants requiring lender consent. Additionally, HTGC shall have the right to participate in a future financing of up to $1,000 under the same terms and conditions and pricing afforded to other participants in that future financing.

In connection with the February 2016 HTGC loan, HTGC also had the right to purchase 80,000 shares of Series C preferred stock at $3.00 per share under the terms of a warrant agreement with the Company. The preferred stock warrant liability was recorded at fair value at the date of issuance of February 17, 2016 in the amount of $134 and recorded as a reduction in the carrying amount of long-term debt on our balance sheet. This discount of $134 will be amortized to interest expense through the loan maturity date of September 1, 2019 using the effective interest

(in thousands, except unit, share, per unit, and per share data)

method. The Company estimated the fair value of the preferred stock warrant liability at the end of each reporting period using the Black-Scholes model and recorded any changes in fair value to other income (expense), net on our statement of operations. See Note 10, *Fair Value Measurements*, for more information on the fair value of the preferred stock warrant liability. The warrant agreement to purchase shares of preferred stock was terminated on September 26, 2017 in connection with the Merger.

At September 30, 2017 and December 31, 2016, the carrying value of long-term debt is $5,221 and $5,667, respectively.

At September 30, 2017, the principal maturities of the long-term debt were as follows:

	September 30, 2017
2017	$ 612
2018	2,620
2019	2,172
Principal balance outstanding	5,404
less: unamortized discount	(164)
less: unamortized debt issuance costs	(19)
Long-term debt	5,221
Current portion	2,551
Noncurrent portion	2,670

The Company paid interest on debt of $158 and $153 during the three months ended September 30, 2017 and 2016, respectively, and $470 and $327 during the nine months ended September 30, 2017 and 2016, respectively.

6. Stockholders' Equity

On September 26, 2017, in connection with the Merger, each share of Exicure OpCo common and preferred stock (other than shares of Exicure OpCo's Series C preferred stock) issued and outstanding immediately prior to the closing of the Merger was converted into 0.49649 shares of Max-1 common stock, and each share of Exicure OpCo's Series C preferred stock issued and outstanding immediately prior to the closing of the Merger was converted into 0.7666652 shares of Max-1 common stock. As a result, an aggregate of 26,666,627 shares of Max-1 common stock were issued to the holders of Exicure OpCo's capital stock, which is incremental to the 2,080,000 shares of Max-1 common stock that were outstanding immediately prior to the Merger. In addition, pursuant to the Merger Agreement options to purchase 7,414,115 shares of Exicure OpCo common stock issued and outstanding immediately prior to the closing of the Merger were assumed by Max-1 and converted into options to purchase 3,680,997 shares of Max-1 common stock.

Common Stock

As of September 30, 2017, the Company had 200,000,000 shares of common stock, par value $0.0001 authorized and 35,513,987 shares issued and outstanding.

The holders of shares of the Company's common stock are entitled to one vote per share on all matters to be voted upon by Exicure stockholders and there are no cumulative rights. Subject to preferences that may be applicable to any outstanding preferred stock, the holders of shares of the Company's common stock are entitled to receive ratably any dividends that may be declared from time to time by Exicure's board of directors (the "Board") out of funds legally available for that purpose. In the event of the Company's liquidation, dissolution or winding up, the holders of shares of Exicure common stock are entitled to share ratably in all assets remaining after payment of liabilities, subject to prior distribution rights of preferred stock then outstanding. Exicure common stock has no

preemptive or conversion rights or other subscription rights. There are no redemption or sinking fund provisions applicable to Exicure common stock. The outstanding shares of Exicure common stock are fully paid and non-assessable.

Preferred Stock

As of September, 30, 2017, the Company had 10,000,000 shares of preferred stock, par value $0.0001 authorized and no shares issued and outstanding.

Private Placement Offering

On September 26, 2017, following the Effective Time of the Merger, the Company sold 6,767,360 shares of Exicure, Inc. common stock pursuant to an initial closing of a private placement offering (the "Offering") for up to 13,333,333 shares of Exicure, Inc. common stock at a purchase price of $3.00 per share (the "Offering Price"). The aggregate net proceeds from the initial closing of the Offering (after deducting placement agent fees and expenses of the initial offering of $3,037) were $17,235. Warrants to purchase 163,174 shares of common stock were issued to the placement agents. The warrants expire on March 27, 2021 and have an exercise price of $3.00 per share. The warrants to purchase common stock are classified as a liability and presented as a dividend that offsets the gross proceeds of the Offering within the accompanying consolidated statement of equity. The common stock warrant liability will be remeasured each period at fair value. See Note 10, *Fair Value Measurements* for more information on the common stock warrant liability.

As discussed in Note 13, *Subsequent Events,* the Company held subsequent closings on October 27, 2017 and November 2, 2017 (the "Subsequent Closings") and sold an aggregate of 3,736,836 shares of Exicure, Inc. common stock in the private placement offering.

Subject to certain customary exceptions, investors in the Offering have anti-dilution protection with respect to the shares of common stock sold in the Offering such that if within eighteen (18) months after the initial closing of the Offering the Company issues certain additional shares of common stock or common stock equivalents for a consideration per share less than the Offering Price (the "Lower Price"), each such investor will be entitled to receive from the Company additional shares of common stock in an amount such that, when added to the number of shares of common stock initially purchased by such investor in the Offering and still held of record and beneficially owned by such investor at the time of the dilutive issuance (the "Held Shares"), will equal the number of shares of common stock that such investor's aggregate purchase price for the Held Shares would have purchased at the Lower Price. Either (i) holders of a majority of the then Held Shares or (ii) a representative of the holders of the then Held Shares, which representative shall be appointed by the three investors who then hold the largest number of Held Shares, may waive the anti-dilution rights of all Offering investors with respect to a particular issuance by the Company.

This price-based anti-dilution protection will automatically terminate upon our receipt of gross proceeds of $40,000 or more in one or more related closings in a bona fide transaction in which we issue shares of common stock or certain common stock equivalents.

The Offering was exempt from registration under Section 4(a)(2) of the Securities Act of 1933, as amended (the "Securities Act"), and Rule 506 of Regulation D promulgated by the SEC. The common stock in the Offering was sold to "accredited investors," as defined in Regulation D, and was conducted on a "reasonable best efforts" basis.

Registration Rights

In connection with the Merger and the Offering, the Company entered into a Registration Rights Agreement, pursuant to which the Company has agreed that promptly, but no later than 60 calendar days from the final closing of the Offering, the Company will file a registration statement with the SEC, or the Registration Statement. Each Investor in the Subsequent Closing also entered into the same registration rights agreement signed by investors in the initial closing of the Offering, which requires that the Company file a "resale" registration statement with the

SEC covering the shares of common stock and warrants issued in the Offering, certain other shares of common stock issued in connection with the Company's recently closed reverse merger, and shares held by the Company's pre-merger stockholders, within 60 calendar days from the final closing of the Offering.

Capitalization Prior to the Merger

All share and per share figures herein are retrospectively adjusted to reflect the conversion of each share of Exicure OpCo common and preferred stock (other than shares of Exicure OpCo's Series C preferred stock), preferred stock warrant liability, and common stock options issued and outstanding immediately prior to the closing of the Merger into 0.49649 shares of the Company's common stock, and each share of Exicure OpCo's Series C preferred stock issued and outstanding immediately prior to the closing of the Merger into 0.7666652 shares of the Company's common stock.

On July 9, 2015, Exicure OpCo converted from a limited liability company into a C corporation. In connection with the corporate conversion, each common unit, Class A unit, Class B-1 unit, Class B-2 unit and Class C unit of AuraSense Therapeutics, LLC issued and outstanding immediately prior to the effectiveness of the corporate conversion was converted into one share of common stock, Series A preferred stock, Series B-1 preferred stock, Series B-2 preferred stock and Series C preferred stock of Exicure OpCo, respectively. Fractional units of AuraSense Therapeutics, LLC that were outstanding prior to the corporate conversion were not converted to shares of stock in Exicure OpCo, resulting in 46 less aggregate shares of outstanding preferred stock of Exicure OpCo as compared to aggregate outstanding preferred units of AuraSense Therapeutics, LLC in connection with the corporate conversion. Each outstanding option to purchase one common unit of AuraSense Therapeutics, LLC was converted into an option to purchase one share of common stock of Exicure OpCo.

Class A Units/Series A Preferred Stock

On December 12, 2011, the Company entered into agreements with AuraSense, LLC: the Bill of Sale, Assignment and Assumption Agreement and the Partial Assignment of License Agreement. In exchange for the contribution of certain assets and agreeing to certain undertakings, the Company provided to AuraSense, LLC 11,381,611 of its Class A units.

For purposes of determining the value of its initial capital contribution, the members of AuraSense Therapeutics, LLC agreed that the Class A units provided to AuraSense, LLC were to be valued at $2.22 per unit or an aggregate value of $25,217. For accounting purposes, however, as the Company and AuraSense, LLC were under common control, the assets and liabilities transferred and the Class A equity contributions were valued at AuraSense, LLC's net book value. The net book value of AuraSense, LLC's contribution at inception was $135.

As further discussed above, in connection with the corporate conversion, 11,381,611 Class A units were converted to 11,381,640 shares of Exicure OpCo Series A preferred stock.

Liquidation preference. The Class A units/Series A preferred stock were senior in right of payment only to the common units until such time as they have received aggregate distributions equal to their unreturned cash contribution.

Class B Units/Series B Preferred Stock

The Class B units/Series B preferred stock were divided into two tranches and have been issued in two series: Class B-1/Series B-1 and Class B-2/Series B-2.

Class B-1/Series B-1. On December 12, 2011, the Company sold 2,451,571 of its Class B-1 units at a price per unit of $2.22. Total gross proceeds raised thereby were $5,432 including a receivable of $500 for 225,677 of the sold units. The receivable was collected in 2012. Net proceeds after associated costs and expenses of $61 were $5,371.

Class B-2/Series B-2. On June 27, 2013, the Company sold 1,403,995 of its Class B-2 units at a price per unit of $2.62. Total gross proceeds raised thereby were $3,676 and proceeds net of $35 in financing costs were $3,641.

As further discussed above, in connection with the corporate conversion, 2,451,571 Class B-1 units and 1,403,995 Class B-2 units were converted to 2,451,560 and 1,403,984 shares of Exicure OpCo Series B-1 and Series B-2 preferred stock, respectively.

Liquidation preference. The Class B units/Series B preferred stock were senior to the Class A units/Series A preferred stock and common units/stock in their right to payment until such time as they have received aggregate distributions equal to their unreturned cash contribution.

Class C Units/Series C Preferred Stock

On June 13, 2014, the Company sold 4,532,112 of its Class C units at a price per unit of $3.00. Total gross proceeds raised thereby were $13,596. Net proceeds after associated costs and expenses of $117 were $13,479.

On February 6, 2015, the Company sold 1,541,168 of its Class C units at a price of $3.00 per unit. Total gross proceeds raised thereby were $4,624. Net proceeds after associated costs and expenses of $43 were $4,581.

As further discussed above, in connection with the corporate conversion, 6,073,280 Class C units were converted to 6,073,226 shares of Exicure OpCo Series C preferred stock.

In October 2015, the Company sold 5,016,134 shares of its Series C preferred stock at a price of $3.00 per share. Total gross proceeds raised thereby were $15,048. Net proceeds after associated costs and expenses of $69 were $14,979.

On January 11, 2016, the Company sold 149,999 shares of its Series C preferred stock at a price of $3.00 per share. Total gross proceeds raised thereby were $450. Net proceeds after associated costs and expenses of $6 were $444.

Liquidation preference. The Class C units/Series C preferred stock were senior to the Class A and Class B units/preferred stock and common units/stock in rights and privileges as established in the Exicure OpCo Operating Agreement. Principal among the rights of Class C units/preferred stock was the creation of the Class C liquidation preference whereby, in the event of a liquidation event (i.e., a liquidation, dissolution or winding up of the Company or a sale of the Company), the Class C preferred unit/stock holders were entitled to receive 1.5 times the aggregate cash contribution of all holders of Class C preferred units/stock.

7. Equity-Based Compensation

On September 22, 2017, the Board adopted and Exicure's stockholders approved the Exicure, Inc. 2017 Equity Incentive Plan (the "2017 Plan"), which is expected to become effective on or about November 15, 2017. The 2017 Plan provides for the issuance of incentive awards of up to 5,842,525 shares of Exicure common stock, which includes 2,158,708 shares of Exicure common stock to be issued to officers, employees, consultants and directors, plus a number of shares not to exceed 3,683,817 that are subject to issued and outstanding awards under the Exicure OpCo 2015 Equity Incentive Plan (the "2015 Plan") and were assumed in the Merger. Awards that may be awarded under the 2017 Plan include non-qualified and incentive stock options, stock appreciation rights, bonus shares, restricted stock, restricted stock units, performance units and cash-based awards. The 2017 Plan also provides that the number of shares reserved for issuance thereunder will be increased annually on the first day of each year beginning in 2020 by the least of 4,600,000 shares, five percent (5%) of the shares of Exicure common stock outstanding on the last day of the immediately preceding year, or a lesser number of shares as determined by the Company's compensation committee. No future awards will be made under the 2015 Plan upon the effectiveness of the 2017 Plan. The aggregate number of common stock options available for grant under the 2017 Plan was 2,158,708 as of September 30, 2017.

(in thousands, except unit, share, per unit, and per share data)

On October 6, 2015, the Exicure OpCo board of directors adopted the 2015 Plan, subject to approval by the Company's stockholders. Such stockholders approval was received on October 13, 2015. The 2015 Plan replaced the 2014 Plan and all outstanding options as of October 13, 2015 became subject to the terms of the 2015 Plan. Prior to October 6, 2015, the Company had granted common unit options to employees, directors, and consultants under the terms of the Equity Incentive Plan which was adopted on September 1, 2011 and subsequently amended and restated on June 13, 2014 (the "2014 Plan"). In connection with the corporate conversion (see Note 1), each outstanding common unit option became an option to purchase one share of Exicure OpCo common stock.

The common stock options are contingent on the participants' continued employment or provision of non-employee services and are subject to forfeiture if employment or continued service terminates for any reason. The initial stock option grant to an employee, director or consultant vests 25% on the first 12-month anniversary of the grant date and vests 1/48th monthly thereafter until fully vested at the end of 48 months. Subsequent stock option grants vest 1/48th monthly until fully vested at the end of 48 months. The term of common stock option grants is ten years unless terminated earlier as described above.

Equity-based compensation expense is classified in the statements of operations as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
Research and development expense	$ 43	$ 40	$ 129	$ 119
General and administrative expense	319	127	975	388
	$ 362	$ 167	$ 1,104	$ 507

Unamortized equity-based compensation expense at September 30, 2017 was $2,352, which is expected to be amortized over a weighted-average period of 2.5 years.

The Company utilizes the Black-Scholes option-pricing model to determine the fair value of common stock option grants. The Black-Scholes option-pricing model was developed for use in estimating the fair value of traded options that have no vesting restrictions and are fully transferable. The model also requires the input of highly subjective assumptions. In addition to an assumption on the expected term of the option grants as discussed below, application of the Black-Scholes model requires additional inputs for which we have assumed the values described in the table below:

	Nine Months Ended September 30,	
	2017	2016
Expected term	5.3 to 6.5 years	5.0 to 6.9 years
Risk-free interest rate	1.97% to 2.17%; weighted avg. 2.07%	1.01% to 1.41%; weighted avg. 1.26%
Expected volatility	80.8% to 83.1%; weighted avg. 81.0%	79.9% to 82.4%; weighted avg. 80.9%
Forfeiture rate	5%	5%
Expected dividend yield	—%	—%

The expected term is based upon the "simplified method" as described in Staff Accounting Bulletin Topic 14.D.2. Currently, the Company does not have sufficient experience to provide a reasonable estimate of an expected term of its common stock options. The Company will continue to use the "simplified method" until there is sufficient experience to provide a more reasonable estimate in conformance with ASC 718-10-30-25 through 30-26. The risk-free interest rate assumptions were based on the U.S. Treasury bond rate appropriate for the expected term in effect

(in thousands, except unit, share, per unit, and per share data)

at the time of grant. The expected volatility is based on calculated enterprise value volatilities for publicly traded companies in the same industry and general stage of development. The estimated forfeiture rates were based on historical experience for similar classes of employees. The dividend yield was based on expected dividends at the time of grant.

The fair value of the underlying common stock and the exercise price for the common stock options granted during the nine months ended September 30, 2017 and 2016 are summarized in the table below:

Common Stock Options Granted During Period Ended:	Fair Value of Underlying Common Stock	Exercise Price of Common Stock Option
Nine months ended September 30, 2017	$4.21	$4.21
Nine months ended September 30, 2016	$1.91 to $2,12; weighted avg. $1.98	$1.91 to $2,12; weighted avg. $1.98

The Company's common stock has not yet been publicly traded, therefore the Company estimates the fair value of its common stock underlying its common stock options. The grant date fair value of the Company's common stock has been determined by the Board exercising their judgment in the consideration of a variety of factors. For financial reporting purposes, the Company has periodically estimated the per share fair value of Exicure OpCo's common stock at various dates using valuations performed in accordance with the guidance outlined in the American Institute of Certified Public Accountants Practice Aid, Valuation of Privately-Held Company Equity Securities Issued as Compensation (Practice Aid). At September 30, 2017, for financial reporting purposes and principally to aid Exicure in the revaluation of certain common stock option awards to non-employees and certain warrant liabilities, Exicure estimated the per share fair value of its common stock to be $3.00, which is the per share price paid by outside investors in the Offering on September 26, 2017.

The weighted-average grant date fair value of common stock options granted in the nine months ended September 30, 2017 and 2016 was $2.92 and $1.53 per common stock option, respectively.

A summary of common stock option activity as of the periods indicated is as follows:

	Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (years)	Aggregate Intrinsic Value (thousands)
Outstanding - December 31, 2016	3,089,352	$ 1.25	8.2	$ 9,143
Granted	657,843	4.21		
Exercised	(58,440)	0.75		
Forfeited	(10,396)	1.00		
Outstanding - September 30, 2017	3,678,359	$ 1.79	7.8	$ 5,257
Exercisable - September 30, 2017	2,131,730	$ 1.37	7.4	$ 3,704
Vested and Expected to Vest - September 30, 2017	3,595,855	$ 1.77	7.7	$ 5,195

The aggregate intrinsic value of common stock options exercised during the nine months ended September 30, 2017 and 2016 was $202 and $48, respectively.

(in thousands, except unit, share, per unit, and per share data)

8. Income Taxes

From inception through July 9, 2015, the Company was a Delaware LLC for federal and state tax purposes and, therefore, all items of income or loss through July 9, 2015 flowed through to the members of AuraSense Therapeutics, LLC. Effective July 9, 2015, the Company converted from an LLC to a C corporation for federal and state income tax purposes.

The Company incurred a pretax loss in each of the three and nine months ended September 30, 2017 and 2016, which consists entirely of loss in the U.S. and resulted in no provision for income tax expense during the periods then ended. The effective tax rate is 0% in each of the three and nine months ended September 30, 2017 and 2016 because the Company has generated tax losses and has provided a full valuation allowance against its deferred tax assets.

9. Loss Per Common Share

Basic loss per common share is calculated by dividing net loss by the weighted-average number of shares of common stock outstanding during the period. Diluted loss per common share is calculated using the treasury share method by giving effect to all potentially dilutive securities that were outstanding. Potentially dilutive options and warrants to purchase common stock that were outstanding during the periods presented were excluded from the diluted loss per share calculation because such shares had an anti-dilutive effect due to the net loss reported in those periods. Therefore, basic and diluted loss per common share is the same for each of the three and nine months ended September 30, 2017 and 2016.

The following is the computation of loss per common share for the three and nine months ended September 30, 2017 and 2016:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
Net loss	$ (2,313)	$ (3,526)	$ (8,937)	$ (11,299)
Weighted-average basic and diluted common shares outstanding	1,725,906	97,691	183,395	109,539
Loss per share - basic and diluted	$ (1.34)	$ (36.09)	$ (48.73)	$ (103.15)

The outstanding securities presented below were excluded from the calculation of net loss per common share, because such securities would have been anti-dilutive due to the Company's net loss per share during the periods ending on the dates presented:

	September 30,	
	2017	2016
Options to purchase common stock	3,678,359	3,038,772
Warrants to purchase common stock	163,174	—

10. Fair Value Measurements

ASC Topic 820, Fair Value Measurement, establishes a three-tier fair value hierarchy, which prioritizes the inputs used in measuring fair value, as follows: Level 1 Inputs - unadjusted quoted prices in active markets for identical assets or liabilities accessible to the reporting entity at the measurement date; Level 2 Inputs - other than quoted prices included in Level 1 inputs that are observable for the asset or liability, either directly or indirectly, for substantially the full term of the asset or liability; and Level 3 Inputs - unobservable inputs for the asset or liability

(in thousands, except unit, share, per unit, and per share data)

used to measure fair value to the extent that observable inputs are not available, thereby allowing for situations in which there is little, if any, market activity for the asset or liability at measurement date.

The Company uses the market approach and Level 1 inputs to value its cash equivalents.

The Company's long-term debt bore interest at the prevailing market rates for instruments with similar characteristics and, accordingly, the carrying value for this instrument also approximates its fair value and the financial measurement is also classified within Level 2 of the fair value hierarchy.

The Company's preferred stock warrant liability and common stock warrant liability are classified within Level 3 of the fair value hierarchy. The fair value of the preferred stock warrant liability and common stock warrant liability was determined using the Black-Scholes option-pricing model.

The fair value of the preferred stock warrant was based significantly on the fair value of the Series C preferred stock, which was developed using unobservable inputs, which are classified within Level 3. At the date of issuance, the preferred stock warrant liability was determined using the following assumptions: expected term of 5.0 years, risk-free interest rate of 1.26%, expected volatility of 62.99%, and no expected dividends. In connection with the Merger, the warrants to purchase preferred stock were terminated and therefore the related liability was reduced to zero.

The fair value of the common stock warrant is based significantly on the fair value of the Company's common stock. At the date of issuance, the common stock warrant liability was determined using the following assumptions: expected term of 2.0 years, risk-free interest rate of 1.44%, expected volatility of 78.71%, and no expected dividends.

The following assumptions were used to estimate the fair value of the common stock warrant liability at September 30, 2017:

	September 30, 2017
Expected term	2.0 years
Risk-free interest rate	1.46%
Expected volatility	78.78%
Expected dividend yield	—%

A 10% change in the estimate of expected volatility at September 30, 2017 would increase or decrease the fair value of the common stock warrant liability in the amount of $18. A 10% change in the estimate of fair value of the common stock at September 30, 2017 would increase or decrease the fair value of the common stock warrant liability in the amount of $34.

The following is a reconciliation of the Company's liabilities measured at fair value on a recurring basis using unobservable inputs (Level 3) for the nine months ended September 30, 2017:

	Fair Value Measurements Using Significant Unobservable Inputs (Level 3)		
	Preferred Stock Warrant Liability	Common Stock Warrant Liability	Total
Balance at January 1, 2017	$ 201	$ —	$ 201
Additions	—	211	211
Loss included in other income (expense), net	(201)	—	(201)
Balance at September 30, 2017	$ —	$ 211	$ 211

(in thousands, except unit, share, per unit, and per share data)

11. Commitments and Contingencies

Leases

The Company conducts all operations in a facility under an operating lease which commenced in March 2012 and was originally scheduled to end in February 2015. During the first quarter of 2014, the lease was extended for an additional six years through February 2021, and includes a renewal option. During the second quarter of 2016, the Company amended the lease agreement to include additional space to be used primarily for administrative functions effective in May 2016. Lease payments include a fixed payment amount as well as contingent payments related to a proportionate share of operating and real estate expenses. At the inception of the lease, the lessor paid for leasehold improvements totaling $52 which has been capitalized and is being amortized over the lease term. The fixed payment amounts, including those in connection with the amended lease agreement in the second quarter of 2016, increase over the term of the lease but rent expense is recognized on a straight-line basis resulting in the recognition of deferred rent liability of $48 and $50 as of September 30, 2017 and December 31, 2016, respectively, calculated on the basis of the extended lease agreement.

Rent expense consisted of the following:

	Three Months Ended September 30,		Six Months Ended September 30,	
	2017	2016	2017	2016
Straight-line rent expense	$ 83	$ 83	$ 249	$ 216
Contingent rent expense	77	83	232	191
Total rent expense	$ 160	$ 166	$ 481	$ 407

Future minimum lease payments as of September 30, 2017 are as follows:

Years ending December 31,	Operating Leases
2017	$ 84
2018	341
2019	347
2020	353
2021	59
Thereafter	—
Total	$ 1,184

Northwestern University license agreements

On December 12, 2011, (1) AuraSense, LLC assigned to the Company all of its worldwide rights and interests under AuraSense, LLC's 2009 license agreement with Northwestern University (“NU”) in the field of the use of nanoparticles, nanotechnology, microtechnology or nanomaterial-based constructs as therapeutics or accompanying therapeutics as a means of delivery, but expressly excluding diagnostics (the “assigned field”); (2) in accordance with the terms and conditions of this assignment, the Company assumed all liabilities and obligations of AuraSense, LLC as set forth in its license agreement in the assigned field; and (3) in order to secure this assignment and the patent rights from NU, the Company agreed (i) to pay NU an annual license fee, which may be credited against any royalties due to NU in the same year, (ii) to reimburse NU for expenses associated with the prosecution and maintenance of the license patent rights, (iii) to pay NU royalties based on any net revenue generated by the Company's sale or transfer of any licensed product, and (iv) to pay NU, in the event the Company grants a sublicense under the licensed patent rights, the greater of a percentage of all sublicensee royalties or a percentage of

(in thousands, except unit, share, per unit, and per share data)

any net revenue generated by a sublicensee's sale or transfer of any licensed product. In August 2015, we entered into a restated license agreement with NU (the "restated license agreement"). In February 2016, we obtained exclusive license as to NU's rights in certain SNA technology we jointly own with NU (the "February 2016 license agreement"). Our license to NU's rights is limited to the assigned field, however we have no such limitation as to our own rights in this jointly owned technology. In June 2016, we entered into an exclusive license with NU to obtain worldwide rights to certain inhibitors of glucosylceramide synthase and their use in wound healing in diabetes (the "June 2016 license agreement"). Our rights and obligations in these 2016 agreements are substantially the same as in the restated license agreement from August 2015 (collectively referred to as "the Northwestern University License Agreements"). As of September 30, 2017, the Company has paid to NU an aggregate of $3,247 in consideration of each of the obligations described above.

12. Related-Party Transactions

Since its inception in 2011, the Company has shared facilities, certain staff members and certain operating expenses with AuraSense, LLC. On an infrequent basis, the Company also pays certain expenses directly on behalf of AuraSense, LLC which are related to AuraSense, LLC's grants, and AuraSense, LLC sometimes pays expenses directly on behalf of the Company. These costs are summarized and directly billed between the Company and AuraSense, LLC on a quarterly basis. In addition, certain expense and administrative activities are shared between the Company and AuraSense, LLC. Effective January 1, 2016, the Company and AuraSense, LLC amended its shared services agreement to simplify the billing arrangement. Under the amended shared services agreement, the Company bills AuraSense, LLC $8 per quarter for indirect costs incurred by the Company plus a specified rate for hours worked by Company scientists on projects directly related to AuraSense, LLC. The amended shared services arrangement continues to require direct non-labor expenses incurred by the Company to be billed to AuraSense, LLC. Effective January 1, 2017, the Company and AuraSense, LLC further amended its shared services agreement so that the quarterly fee related to administrative activities billed by the Company to AuraSense, LLC be reduced to $3 per quarter. This decrease is to reflect the current and expected future reduction in administrative activities to be provided by the Company to AuraSense, LLC.

The amounts due from AuraSense, LLC in connection with the above mentioned activities were $13 and $15 at September 30, 2017 and December 31, 2016, respectively.

The following is a summary of amounts billed to AuraSense, LLC and recognized in the accompanying unaudited statement of operations in connection with the above mentioned activities:

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2017	2016	2017	2016
Direct labor on research activities	$ —	$ —	$ —	$ 2
Quarterly fee for indirect costs	3	8	9	23
Direct costs of AuraSense LLC paid by the Company or (of the Company paid by AuraSense, LLC), net	1	—	4	(4)
	$ 4	$ 8	$ 13	$ 21

The Company received consulting services from, and paid fees to, one of its co-founders who is not an employee but serves as a member of the Board of Directors of the Company. The Company paid $75 in each of the nine months ended September 30, 2016 and 2017 in connection with these consulting services and these amounts are recognized as an expense in the accompanying unaudited consolidated statement of operations.

13. Subsequent Events

Private placement - subsequent closings

On October 27, 2017 and November 2, 2017, Exicure entered into subscription agreements (the "Subscription Agreements") with several accredited investors (the "Investors") pursuant to which the Company agreed to issue and sell a total of 3,736,836 shares of the Company's common stock, par value $0.0001 per share (the "Shares") resulting in approximately $11,211 in gross proceeds to the Company. These shares were issued in Subsequent Closings of the Offering for up to 13,333,333 shares of common stock (the "Maximum Amount") at a purchase price of $3.00 per share (the "Sale Price"). The Company and Katalyst Securities LLC, a U.S. registered broker-dealer (together with its sub-agents, the "Placement Agents") may agree to extend the period for additional closings up to November 30, 2017.

As of the date of this Quarterly Report on Form 10-Q, the Company has sold a total of 10,504,196 shares of common stock for a total of approximately $31,513 in connection with all closings of the Offering (before deducting placement agent fees and expenses which are estimated at $3,966). Placement Agents have received an aggregate of $1,968 in cash placement fees and will receive warrants to purchase an aggregate of 413,320 shares of Exicure common stock (the "Warrants") in connection with all closings of the Offering as of the date of this Quarterly Report on Form 10-Q. The Warrants have an exercise price of $3.00 per share and have been issued on the same terms in all prior closings of the Offering. The Placement Agents will also receive 50,000 shares of Exicure common stock in connection with all closings of the Offering.

The Company has evaluated subsequent events which may require adjustment to or disclosure in the financial statements through the date the financial statements were issued.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations.

You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and the related notes and other financial information included in this Quarterly Report on Form 10-Q. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report on Form 10-Q, including information with respect to our plans and strategy for our business, includes forward-looking statements that involve risks and uncertainties as described under the heading "Forward-Looking Statements" elsewhere in this Quarterly Report on Form 10-Q. You should review the disclosure under the heading "Risk Factors" in this Quarterly Report on Form 10-Q for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Operating Overview

We are a clinical-stage biotechnology company developing gene regulatory and immuno-oncology therapeutics based on our proprietary SNA, technology. We believe the design of our SNAs gives rise to distinct chemical and biological properties that may provide advantages over other nucleic acid therapeutics and enable therapeutic activity outside of the liver. Since our SNAs can cross biological barriers such as the skin when administered locally, we believe they can be used to target diseases not typically addressed with other nucleic acid therapeutics. We have demonstrated the ability to cross biological barriers in a Phase 1 clinical trial of our lead therapeutic candidate, AST-005, and in preclinical studies of two other therapeutic candidates, XCUR17 and AST-008, both of which we are advancing to the clinic.

In a completed Phase 1 clinical trial, AST-005, when topically administered to the skin of patients with mild to moderate psoriasis, resulted in no drug associated adverse events, and demonstrated a reduction of tumor necrosis factor messenger RNA ("TNF mRNA"). The TNF mRNA reduction elicited by the highest strength of AST-005 gel was statistically significant when compared to the effects of the vehicle. These results are significant because we believe this is the first time gene regulation with topically applied oligonucleotides has been observed in the clinic. While we did not observe an antipsoriatic effect in our Phase 1 clinical trial, we believe this is due to the short duration of the treatment. The results of a clinical trial with etanercept, a systemic TNF inhibitor, indicate that at least four weeks of therapy is required before antipsoriatic efficacy can be observed.

On December 2, 2016, we entered into a research collaboration, option and license agreement with Purdue referred to as the Purdue Collaboration. Purdue has the option to obtain from us the full worldwide development and commercial rights to AST-005, an option to obtain three additional collaboration targets and a further option to obtain from us the full worldwide development and commercial rights to any therapeutic candidates developed targeting the three additional collaboration targets. Additionally, Purdue has rights of first offer to some potential collaboration targets. These rights of first offer are subject to limitations in time and scope. In connection with the Purdue Collaboration, we received a non-refundable development fee of $10 million. In addition, we are eligible to receive up to $776.5 million upon successful completion of certain research, regulatory and commercial sales milestones. We cannot assure you that these milestones will be achieved as they are subject to highly significant risks and uncertainties, many of which are outside of our control. In the event a therapeutic candidate subject to the collaboration results in commercial sales, we are eligible to receive royalties ranging from the low single digits to a maximum of 10% on future net sales of such commercialized therapeutic candidates. We are conducting, on behalf of Purdue, a Phase 1b clinical trial in psoriasis patients in Germany to evaluate the effect of higher concentrations of AST-005 gel on TNF mRNA and downstream mRNA expression. We expect the results from this clinical trial to be available in late 2017.

Our second therapeutic candidate, XCUR17, is an SNA targeted to IL-17RA for the treatment of mild to moderate psoriasis. We filed a CTA for a Phase 1 clinical trial of XCUR17 in patients with psoriasis in Germany in the third quarter of 2017 and expect to commence the Phase 1 clinical trial in the fourth quarter of 2017. We expect the results from this clinical trial to be available in the second quarter of 2018. Our third therapeutic candidate, AST-008, is an SNA consisting of toll-like receptor 9 ("TLR9") agonists designed for immuno-oncology applications. AST-008 has exhibited anti-tumor activity as both a monotherapy and in combination with certain checkpoint inhibitors across a range of preclinical models of solid and hematological cancers. In the third quarter of

2017, we received an authorization from the Medicines and Healthcare products Regulatory Agency (the "MHRA") to conduct a Phase 1 clinical trial with AST-008 in the United Kingdom. Although we plan to begin AST-008 clinical development as a monotherapy in a Phase 1 clinical trial in the fourth quarter of 2017, we ultimately plan to clinically advance AST-008 exclusively in combination with checkpoint inhibitors. We expect the results from the Phase 1 clinical trial to be available on a rolling basis starting in the fourth quarter of 2017.

Since our inception in 2011, we have devoted substantial resources to the research and development of SNAs and the protection and enhancement of our intellectual property. We have no products approved for sale and all of our $13.4 million in revenue through September 30, 2017 has been earned through our research collaboration, license, and option agreement with Purdue or as a primary contractor or as a subcontractor on government grants. In addition to our revenue, through September 30, 2017, we have funded our operations through private placements of preferred stock with gross proceeds totaling $42.8 million, sales of common stock in an initial closing of the Offering with gross proceeds totaling $20.3 million and debt financing totaling $6.0 million. As of September 30, 2017, our cash and cash equivalents were $22.9 million.

Since our inception, we have incurred significant operating losses. Our net loss was $2.3 million and $3.5 million for the three months ended September 30, 2017 and 2016, respectively, and $8.9 million and $11.3 million for the nine months ended September 30, 2017 and 2016, respectively. As of September 30, 2017, our accumulated deficit was $50.4 million. Substantially all of our operating losses resulted from expenses incurred in connection with our research programs and from general and administrative costs associated with our operations.

We expect to continue to incur significant and increasing losses in the foreseeable future. Our net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially as we:

- conduct further preclinical studies and clinical trials of AST-008 and XCUR17;
- increase research and development for the discovery and development of additional therapeutic candidates;
- advance other therapeutic candidates into preclinical and clinical development;
- increase our research and development to enhance our technology;
- procure clinical trial materials;
- seek regulatory approval for our therapeutic candidates that successfully complete clinical trials;
- maintain, expand and protect our intellectual property portfolio;
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts; and
- operate as a public company.

We have not generated any commercial product revenue nor do we expect to generate substantial revenue from product sales unless and until we successfully complete development and obtain regulatory approval for one or more of our therapeutic candidates. Successful therapeutic development and regulatory approval are subject to significant uncertainty and we expect will take at least five years. If we obtain regulatory approval for any of our therapeutic candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution. Other sources of revenue could include a combination of research and development payments, license fees and other upfront payments, milestone payments, and royalties in connection with our current and any future collaborations and licenses. Until such time, if ever, that we generate revenue from whatever source, we expect to finance our cash needs through a combination of public or private equity offerings, debt financings and research collaboration and license agreements. We may be unable to raise capital or enter into such other arrangements when needed or on favorable terms. Our failure to raise capital or enter into such other arrangements as and when needed would have a negative impact on our financial condition and our ability to develop our therapeutic candidates.

Recent Developments

Reverse Merger

On September 26, 2017, pursuant to the Merger Agreement, Max-1 Acquisition Sub, Inc., a wholly-owned subsidiary of Max-1, merged with and into Exicure, Inc. (formerly AuraSense Therapeutics, LLC), a privately held Delaware corporation referred to herein as Exicure OpCo., with Exicure OpCo remaining as the surviving entity and a wholly-owned operating subsidiary of Max-1. The Merger was effective as of September 26, 2017 (the "Effective Time"), upon the filing of a Certificate of Merger with the Secretary of State of the State of Delaware.

At the Effective Time, the legal existence of Max-1 Acquisition Sub, Inc. ceased. At the Effective Time, each share of Exicure OpCo common and preferred stock (other than shares of Exicure OpCo's Series C preferred stock) issued and outstanding immediately prior to the closing of the Merger was converted into 0.49649 shares of Max-1 common stock, and each share of Exicure OpCo's Series C preferred stock issued and outstanding immediately prior to the closing of the Merger was converted into 0.7666652 shares of Max-1 common stock. As a result, an aggregate of 26,666,627 shares of Max-1 common stock were issued to the holders of Exicure OpCo's capital stock, which is incremental to the 2,080,000 shares of Max-1 common stock that were outstanding immediately prior to the Merger. In addition, pursuant to the Merger Agreement options to purchase 7,414,115 shares of Exicure OpCo common stock issued and outstanding immediately prior to the closing of the Merger were assumed and converted into options to purchase 3,680,997 shares of Max-1 common stock. After the filing of the Certificate of Merger with the Secretary of State of the State of Delaware, Max-1 changed its name to Exicure, Inc.

The Merger is considered a "reverse merger", whereby Exicure OpCo is considered the accounting acquirer in the Merger. Exicure OpCo was determined to be the accounting acquirer based on the terms of the Merger and other factors including: (i) legacy Exicure OpCo shareholders own approximately 94% of the combined company on a fully diluted basis immediately following the closing of the Merger, (ii) legacy Exicure OpCo directors will hold all six board seats of the combined company, and (iii) legacy Exicure OpCo management will hold all key positions in management of the combined company. The transaction is accounted for as an asset acquisition rather than a business combination because as of the acquisition date, Max-1 did not meet the definition of a business as defined by U.S. GAAP. Consequently, the assets, liabilities and operations that are reflected in Exicure's historical financial statements prior to the Merger will be those of Exicure OpCo, and the consolidated financial statements after completion of the Merger will include the assets, liabilities and results of operations of Exicure OpCo up to the day prior to the closing of the Merger and the assets, liabilities and results of operations of the combined company from and after the closing date of the Merger. The assets and liabilities of Max-1 included in the accompanying consolidated financial statements are recorded at the historical cost basis of Max-1.

Private Placement

Following the Effective Time of the Merger, we held several closings of the Offering on September 26, 2017, October 27, 2017 and November 2, 2017 in which we sold to accredited investors approximately $31.5 million worth of shares of common stock (before deducting placement agent fees and expenses which are estimated at approximately $4.0 million), or 10,504,196 shares, at a price of $3.00 per share. Also, we granted the investors in the Offering registration rights requiring us to register those shares of common stock for public resale. The then existing stockholders of Exicure OpCo who agreed to become parties to the registration rights agreement also became entitled to such registration rights.

The Company and Katalyst Securities LLC, a U.S. registered broker-dealer (together with its sub-agents, the "Placement Agents") may agree to extend the period for additional closings up to November 30, 2017.

Each investor in any Subsequent Closing of the Offering was required to represent that, at the time of the applicable closing, it (i) had a substantive, pre-existing relationship with us, or had direct contact with the Company or the Placement Agent or other enumerated parties outside of the Offering, (ii) was not identified or contacted through the marketing of the Offering, and (iii) did not independently contact us as a result of general solicitation by means of our Current Report on Form 8-K filed with the SEC on October 2, 2017, as amended (the "Form 8-K"), any press release or any other public disclosure disclosing the material terms of the Offering.

Segment Reporting

We view our operations and manage our business as one segment, which is the discovery, research and development of treatments based on our SNA technology.

Critical Accounting Policies and Significant Judgments and Estimates

Our management's discussion and analysis of financial condition and results of operations is based on our financial statements, which have been prepared in accordance with GAAP. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the revenue and expenses incurred during the reported periods. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not apparent from other sources. Changes in estimates are reflected in reported results for the period in which they become known. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in the notes to our financial statements appearing in this Report, we believe that the following critical accounting policies are most important to understanding and evaluating our reported financial results.

Revenue recognition

We recognize revenue when the following criteria have been met: (1) persuasive evidence of an arrangement exists; (2) delivery has occurred or services have been rendered and risk of loss has passed; (3) the fee is fixed or determinable; and (4) collectability is reasonably assured. When payments are received in advance of recognizing revenue, we include the amount in deferred revenue on the balance sheet. Amounts deferred that are not anticipated to be recognized as revenue within a year of the balance sheet date are classified as noncurrent liabilities.

We have generated all of our revenue to date through our research collaboration, license, and option agreement with Purdue or as a primary contractor or as a subcontractor on government grants. We have not generated any commercial product revenue. Historically, our research collaborations and grants have been either as a direct contractor or as a sub-awardee on contracts funded by various governmental agencies.

In arrangements involving the delivery of more than one element, each required deliverable is evaluated to determine whether it qualifies as a separate unit of accounting. The determination is based on whether the deliverable has "standalone value" to the customer. If a deliverable does not qualify as a separate unit of accounting, it is combined with the other applicable undelivered item(s) within the arrangement and these combined deliverables are treated as a single unit of accounting.

The arrangement's consideration that is fixed or determinable is allocated to each separate unit of accounting based on the relative selling price methodology in accordance with the selling price hierarchy, which includes vendor-specific objective evidence ("VSOE"), of selling price, if available, or third-party evidence of selling price if VSOE is not available, or the best estimate of selling price, if neither VSOE nor third-party evidence is available.

Payments or reimbursements for our research and development efforts for the arrangements where such efforts are considered as deliverables are recognized as the services are performed and are presented on a gross basis. When upfront payments are received and if there is no discernible pattern of performance and/or objectively measurable performance measures do not exist, we recognize revenue ratably over the associated period of performance.

Purdue Collaboration

On December 2, 2016, Exicure entered into a research collaboration, option and license agreement with Purdue and referred to as the Purdue Collaboration. Purdue has the option to obtain from us the full worldwide development and commercial rights to AST-005 (the Company's lead therapeutic candidate that targets tumor necrosis factor), an option to obtain three additional collaboration targets and a further option to obtain from us the full worldwide development and commercial rights to any therapeutic candidates developed targeting the three additional collaboration targets. Additionally, Purdue has rights of first offer to some potential collaboration targets. These rights of first offer are subject to limitations in time and scope. In connection with the Purdue Collaboration, we received a non-refundable development fee of $10.0 million. In addition, we are eligible to receive up to $776.5 million upon successful completion of certain research, regulatory and commercial sales milestones. The research milestones are payable upon target identification and IND-enabling pre-clinical development, per program, with an aggregate total of up to $16.5 million. The regulatory milestones are payable upon the initiation or completion of clinical trials, and regulatory approval in the United States and outside the United States, per program, with an aggregate total of up to $410.0 million. The commercial sales milestones are payable upon achievement of specified aggregate product sales thresholds and total up to $350.0 million. In the event a therapeutic candidate subject to the collaboration results in commercial sales, the Company is eligible to receive royalties ranging from the low single digits to a maximum of 10% on future net sales of such commercialized therapeutic candidates. Additionally, Purdue had an obligation to invest in a qualified equity financing of the Company if such financing was completed before June 2, 2017. We did not complete such qualified equity financing before June 2, 2017.

In accordance with ASC 605-25, we identified the following deliverables at the inception of the Purdue Collaboration agreement: (1) exclusive rights to the TNF-α target, (2) the obligation to participate in a joint research committee, (3) the provision of research and development activities based on a prescribed full-time employee rate per year, (4) a non-voting board of director observer role, (5) Purdue's right to participate in a future qualified equity financing of the Company if such financing occurs prior to June 2, 2017 or the pricing of the initial public offering of shares of the Company's common stock, (6) the option for an exclusive development and commercialization license to AST-005 or a TNF-α development candidate other than AST-005, (7) the option to select and develop three additional collaboration targets, and (8) the option for an exclusive development and commercialization license to any developed therapeutic candidate targeting the three additional collaboration targets. We determined that deliverables (2), (4), (5), and (6) do not have stand-alone value to Purdue, and accordingly, deliverables (2), (4), (5) and (6) were combined with deliverables (1) and (3) as a single unit of accounting. We concluded that, at the inception of the agreement, deliverables (7) and (8) are substantive options and do not contain a significant or incremental discount; as a result, no portion of the upfront $10.0 million is allocated to deliverables (7) and (8).

The upfront payment of $10.0 million was allocated to the single unit of accounting consisting of deliverables (1), (2), (3), (4), (5), and (6) above and was recorded as deferred revenue and is being recognized on a ratable basis over the estimated performance period of the relevant research and development activities of 14.5 months.

During the three months ended September 30, 2017, we recognized collaboration revenue of $2.5 million, which included $0.4 million of research and development activities that will be reimbursed by Purdue and is presented on a gross basis in the accompanying statement of operations. During the nine months ended September 30, 2017, we recognized collaboration revenue of $7.6 million, which included $1.4 million of research and development activities that will be reimbursed by Purdue and is presented on a gross basis in the accompanying statement of operations. As of September 30, 2017 and December 31, 2016, deferred revenue relating to the Purdue Collaboration was $3.1 million and $9.3 million, respectively, of which $3.1 million and $8.3 million, respectively, is classified as current portion of deferred revenue in the accompanying balance sheet.

The Purdue Collaboration agreement includes contingent payments related to specified research, development and regulatory milestones and sales-based milestones. Each contingent and milestone payment is evaluated to determine whether it is substantive and at risk to both parties. We recognize any payment that is contingent upon the achievement of a substantive milestone entirely in the period in which the milestone is achieved. Any payments that are contingent upon achievement of a non-substantive milestone are recognized as revenue prospectively, when such payments become due and collectible, over the remaining expected performance period under the arrangement,

which is generally the remaining period over which the research and development services are expected to be provided. To date, we have not recognized any contingent payments in connection with the Purdue Collaboration as revenue.

Our grant contracts have typically been cost or cost-plus fee contracts. Revenues on these contracts are recognized as costs are incurred, generally based on allowable costs incurred during the period, plus any recognizable earned fee. We consider fixed fees under cost and cost-plus fee contracts to be earned in proportion to the allowable costs incurred in performance of the contract. We analyze costs for contracts and reimbursable grants to ensure that reporting of revenues on a gross basis instead of on a net basis is appropriate.

We have entered into certain grant contracts containing milestone payments. We recognize revenue from milestone payments when earned, provided that the milestone event was substantive, its achievability was not reasonably assured at the inception of the agreement, we have no further performance obligations relating to the event and collectability is reasonably assured. If these criteria are not met, we recognize milestone payments ratably over the remaining period of our performance obligations under the grant contract. For a milestone to be considered substantive, the payment associated with its achievement must have all of the following characteristics: (1) relate solely to past performance; (2) be reasonable, relative to all of the deliverables and payment terms within the arrangement; and (3) be commensurate with either our efforts required to achieve the milestone or the enhanced value of the delivered items(s) as a result of the milestone achievement.

As of September 30, 2017 and December 31, 2016, and for the three and nine months ended September 30, 2017 and 2016, the Company was not participating in any grant research activities involving milestone payments.

Equity-based compensation

We measure the cost of common stock option awards at fair value and record the cost of the awards, net of estimated forfeitures, on a straight-line basis over the requisite service period. We measure fair value for all common stock options using the Black-Scholes option-pricing model. For all common stock option awards to employees, the fair value measurement date is the date of grant and the requisite service period is the period over which the employee is required to provide service in exchange for the common stock option awards, which is generally the vesting period. For all common stock option awards to nonemployees, we remeasure fair value at each financial statement reporting date and recognize compensation expense as services are rendered, generally on a straight-line basis.

In connection with the Merger, Exicure OpCo's outstanding common stock options were assumed and converted into Exicure common stock options.

The Black-Scholes option-pricing model requires the input of highly subjective assumptions, including: (1) the estimated grant date fair value of Exicure's or Exicure OpCo's (as the case may be) common stock; (2) the option exercise price; (3) the expected term of the option in years; (4) the annualized volatility of the stock; (5) the risk-free interest rate; and (6) the annual rate of quarterly dividends on the stock. We make those estimates as follows:

Fair value of Exicure OpCo's common stock. Exicure OpCo's common stock has not yet been publicly traded, therefore we estimate the fair value of the common stock underlying our stock options. The grant date fair value of Exicure OpCo's common stock has been determined by its board of directors exercising their judgment in the consideration of a variety of factors. Please see "—Exicure OpCo common stock valuation" below for a detailed discussion of the assumptions used in estimating the grant date fair value of the common stock underlying Exicure OpCo's stock options. For financial reporting purposes and principally to aid Exicure OpCo in the revaluation of certain common stock option awards to non-employees and certain warrant liabilities, Exicure OpCo has periodically estimated the per share fair value of its common stock at various dates using valuations performed in accordance with the guidance outlined in the American Institute of Certified Public Accountants Practice Aid, Valuation of Privately-Held Company Equity Securities Issued as Compensation (the "Practice Aid"). Exicure OpCo performed these valuations as of the following dates: March 31, 2012; July 31, 2013; June 30, 2014; December 31, 2014; March 31, 2015; June 30, 2015; September 30, 2015; December 31, 2015; March 31, 2016; June 30, 2016; October 31, 2016; December 15, 2016; March 31, 2017; and June 30, 2017.

Fair value of Exicure's common stock. Exicure's common stock has not yet been publicly traded, therefore we estimate the fair value of the common stock underlying our stock options. At September 30, 2017, for financial reporting purposes and principally to aid Exicure in the revaluation of certain common stock option awards to non-employees and certain warrant liabilities, Exicure estimated the per share fair value of its common stock to be $3.00, which is the per share price paid by outside investors in the Offering on September 26, 2017.

Option exercise price. For all common stock options granted prior to August 31, 2013, the grant date exercise price was equal to $2.22 per share based on the purchase price per share of Exicure OpCo's Series B-1 preferred stock sold in December 2011. For all common stock options granted between August 31, 2013 and October 2, 2014, the grant date exercise price was equal to $2.62 per share based on the purchase price per share of Exicure OpCo's Series B-2 preferred stock sold in June 2013. On October 3, 2014, Exicure OpCo's board of directors determined that the fair value of Exicure OpCo's common stock was $0.64 per share and further determined that for all periods prior to October 3, 2014, the fair value of Exicure OpCo's common stock was less than or equal to $0.64 per share. Also on October 3, 2014, Exicure OpCo's board of directors took action by way of unanimous written consent to modify the exercise price on all outstanding stock options to $0.64 to reflect the board's determination of fair value of Exicure OpCo's common stock for all periods prior to such date. No other terms of the repriced stock options were modified and these repriced stock options continued to vest according to their original vesting schedules and retained their original expiration dates.

After the October 3, 2014 modification, Exicure OpCo granted additional common stock options as follows.

Grant Date	Options Granted	Exercise Price	Fair Value of Common Stock at Grant Date
10/3/2014	637,570	$ 0.64	$ 0.64
10/17/2014	24,824	$ 0.64	$ 0.64
4/28/2015	243,273	$ 1.03	$ 1.03
5/14/2015	4,964	$ 1.03	$ 1.03
5/21/2015	4,964	$ 1.03	$ 1.03
5/22/2015	54,612	$ 1.03	$ 1.03
11/24/2015	1,045,669	$ 1.97	$ 1.97
5/11/2016	17,376	$ 1.91	$ 1.91
8/10/2016	7,447	$ 2.11	$ 2.11
11/9/2016	62,059	$ 2.42	$ 2.42
1/4/2017	657,841	$ 4.21	$ 4.21

No additional common stock options were granted since January 4, 2017.

Expected term. In determining the expected term of the options, Exicure and Exicure OpCo elected to use the "simplified" method in accordance with SEC SAB No. 107, Share-Based Payments. The use of the "simplified" method under SAB No. 107 was extended beyond December 31, 2007, in accordance with SAB No. 110, Share-Based Payment, issued on December 21, 2007, until such time when there is sufficient information to make more refined estimates on the estimated life of options. SAB No. 110 expressed the views of the staff regarding the use of the "simplified" method, as discussed in SAB No. 107, in developing an estimate of the expected term of "plain vanilla" share options in accordance with ASC 718, Share-Based Payment. SAB No. 110 allows companies that do not have historically sufficient experience to provide a reasonable estimate to continue use of the "simplified" method for estimating the expected term of "plain vanilla" share options grants after December 31, 2007. The Company will continue to use the "simplified" method until we have enough historical experience to provide a reasonable estimate of expected term in accordance with SAB No. 110.

Volatility. Because Exicure's and Exicure OpCo's common stock has not been freely traded in any market or public exchange, we do not have information on the trading volatility of Exicure's and Exicure OpCo's common

stock. Consequently, we have derived our assumption for volatility by examining the historical volatilities of several unrelated public companies within our industry. When selecting the industry peer group of companies to be used in the volatility calculation, we also consider the stage of development, size and financial leverage of each potential comparable company.

Risk-free rate. The risk-free interest rate is based on the yields of U.S. Treasury securities with maturities similar to the expected term of the options for each option grant.

Dividend yield. Neither Exicure or Exicure OpCo have declared or paid cash dividends and we do not presently plan to pay cash dividends in the foreseeable future. Consequently, we used an expected dividend yield of zero.

We are also required to estimate forfeitures at the time of grant and revise those estimates in subsequent periods if actual forfeitures differ from estimates. We use historical data to estimate pre-vesting option forfeitures and record stock-based compensation expense only for those awards that are expected to vest. Stock-based compensation expense recognized in the financial statements is based on awards that are ultimately expected to vest.

Exicure OpCo common stock valuation

Exicure OpCo has historically granted common stock options at exercise prices not less than the fair value of its common stock. As there has been no public market for Exicure OpCo's common stock to date, the estimated fair value of its common stock has been determined by its board of directors.

On each occasion when Exicure OpCo's board determined the fair value of its common stock, they considered each of the factors listed below as well as certain qualitative factors particular to each grant:

- the prices of Exicure OpCo's preferred stock sold to or exchanged between outside investors in arm's length transactions, and the rights, preferences and privileges of Exicure OpCo's preferred stock as compared to those of Exicure OpCo's common stock, including the liquidation preferences of Exicure OpCo's preferred stock;
- Exicure OpCo's results of operations, financial position and the status of research and development efforts;
- Exicure OpCo's limited capital resources and the risks inherent in raising additional financing;
- the composition of, and changes to, Exicure OpCo's management team and board of directors;
- the lack of liquidity of Exicure OpCo's common stock as a private company;
- Exicure OpCo's stage of development and business strategy and the material risks related to Exicure OpCo's business and industry;
- the achievement of enterprise milestones, including entering into collaboration and license agreements;
- the valuation of publicly traded companies considered to be similar in industry and/or business model to Exicure OpCo as well as recently completed mergers and acquisitions of companies similar in industry and/or business model to Exicure OpCo;
- any external market conditions affecting the global synthetic biology market, the global market for cancer therapeutics, and the global market for nanoparticles in biotechnology and pharmaceuticals;
- the likelihood of achieving a liquidity event for the holders of Exicure OpCo's common stock and stock options, such as an initial public offering or a sale of Exicure OpCo, given prevailing market conditions;
- the state of the initial public offering market for similarly situated privately held biotechnology companies; and
- any recent contemporaneous valuations prepared by Exicure OpCo's board of directors and management in accordance with methodologies outlined in the Practice Aid.

We have periodically determined, for financial reporting purposes, the estimated per share fair value of Exicure OpCo's common stock at various dates using contemporaneous and retrospective valuations. These valuations have been performed in accordance with the guidance outlined in the Practice Aid.

Common stock valuation methodologies. These contemporaneous and retrospective valuations discussed below were prepared in accordance with the guidelines in the Practice Aid, which prescribes several valuation approaches for setting the value of an enterprise, such as the cost, market and income approaches, and various methodologies for allocating the value of an enterprise to its common units. We generally used the income and market approaches. When applying the market approach, we used the guideline company and precedent transaction methodologies based on inputs from comparable public companies' equity valuations and comparable acquisition transactions to estimate Exicure OpCo's enterprise value. In applying the income approach, we applied the discounted cash flow method based on Exicure OpCo's projections.

In order to determine the fair market value of Exicure OpCo's common stock, Exicure OpCo's board of directors first valued the enterprise and then allocated that value through the capital structure.

Methods used to determine the enterprise value of Exicure OpCo. Exicure OpCo's board considered three basic approaches to determining value as outlined in the Practice Aid: 1) Market Approach, 2) Income Approach, and 3) Asset-Based or Cost Approach.

Market Approach. The Market Approach relies on an analysis of publicly traded companies similar in industry and/or business model to Exicure OpCo. This methodology uses these guideline companies to develop relevant market multiples and ratios, using metrics such as revenue, earnings before interest and taxes ("EBIT"), earnings before interest, taxes, depreciation and amortization ("EBITDA"), net income and/or tangible book value. These multiples and values are then applied to Exicure OpCo's corresponding financial metrics. Since no two companies are perfectly comparable, premiums or discounts may be applied to the subject company's metrics if its position in its industry is significantly different from the position of the guideline companies, or if its intangible attributes are significantly different.

Application of the Market Approach will also consider the actual prices paid in merger and acquisition transactions for companies similar to Exicure OpCo. Exit multiples of total purchase price paid to revenue, EBIT, EBITDA, net income and/or tangible book value may be developed for each comparable transaction, if the data is available. These multiples are then applied to Exicure OpCo's corresponding latest 12-month and projected financial metrics.

New to the Practice Aid is the "Back-solve Method." The Back-solve Method, a form of the Market Approach to valuation, derives the implied equity value for one type of equity security (e.g., common stock) from a contemporaneous transaction involving another type of equity security (e.g., preferred stock). The Back-solve Method relies on the Black-Scholes Option pricing model to determine the value of the common stock. Inputs into the Black-Scholes model were determined by an analysis of publicly traded companies similar in industry and/or business model to Exicure OpCo.

During the periods under consideration, Exicure OpCo raised preferred stock financing in arm's length market transactions on six occasions: December 2011, June 2013, June 2014, February 2015, October 2015, and January 2016. All of Exicure OpCo's preferred stock financings included independent, third-party investors and therefore we believe are reliable indications of the fair value for the class of preferred stock acquired at the time. We also believe they form a reasonable basis for estimating the fair value of Exicure OpCo's common stock. In the board's judgment, the proximity between these financings and the date for which a fair market value determination of Exicure OpCo's common stock was required, indicate that the Back-solve Method is the most appropriate method for determining fair market value of Exicure OpCo's common stock.

Income Approach. Exicure OpCo's board determined that the Income Approach cannot be appropriately applied to Exicure OpCo. Exicure OpCo is at a very early stage in its development and is not currently generating material positive cash flow nor do we anticipate generating material positive cash flows within the economic time horizon typically considered in a discounted cash flow analysis. As discussed elsewhere in this Quarterly Report on Form 10-Q, Exicure OpCo is an early stage biotechnology company and Exicure OpCo's primary activities to date have

been research and development of potential new therapeutics based upon its technology. We believe that any valuation of Exicure OpCo's common stock's fair value must recognize that Exicure OpCo is subject to a risky and lengthy period of time until commercial product revenue, if any, is earned. Exicure OpCo's future business model is also uncertain and could include a variety of technology and product licensing opportunities in addition to commercial product revenue from product sales. As a consequence, Exicure OpCo's board has determined that the projection of typical inputs into the Income Approach such as market size, market share, product revenue and the future expense and capital expenditures to support that forecast revenue, cannot be reasonably generated by Exicure OpCo at this time.

Cost Approach. The Cost Approach involves identifying Exicure OpCo's significant tangible assets, estimating the individual current market values of each and then totaling them to derive the value of the business as a whole. The board determined that, based on Exicure OpCo's industry and stage of development, Exicure OpCo's tangible assets are not a reasonable estimation of Exicure OpCo's enterprise value.

Methods used to allocate Exicure OpCo's enterprise value to each class of securities. In accordance with the Practice Aid, we considered the various methods for allocating the enterprise value across Exicure OpCo's classes and series of capital stock to determine the fair value of Exicure OpCo's common stock at each valuation date. The methods we considered consisted of the following:

Current Value Method. Under the Current Value Method, once the fair value of the enterprise is established, the value is allocated to the various series of preferred and common stock based on their respective seniority, liquidation preferences or conversion values, whichever is greatest. We believe that application of the Current Value Method is generally only appropriate when a liquidity event in the form of an acquisition or dissolution of the company is imminent and expectations about the future value of the company are virtually irrelevant. Alternatively, the Current Value Method can be used when the company is so early in its development that no significant economic value has yet been created above the value of the capital invested in the business. We believe neither circumstance fits Exicure OpCo and so we did not use this method in allocating fair value to each of Exicure OpCo's classes of stock.

Option Pricing Method. Prior to March 31, 2015, the principal method by which we allocated value to each class of preferred stock and to the common stock was the Option Pricing Method. Subsequent to March 31, 2015 the Option Pricing Method was used in combination with the Probability Weighted Expected Return method (see below) using a Hybrid Method (see below). Under the Option Pricing Method, shares are valued by creating a series of call options with exercise prices based on the liquidation preferences and conversion terms of each equity class. The values of the preferred and common stock are inferred by analyzing these options. Because of the various liquidation preferences granted to Exicure OpCo's different classes of preferred stock, we utilized a lattice-based option-pricing model. We utilized a twenty-five step binomial lattice in order to estimate a wide range of possible future values for Exicure OpCo's total enterprise value. The binomial lattice is based on the following parameters:

a) dividend yield

b) risk-free rate

c) volatility

d) time to maturity

e) initial firm value

The resultant values at the end nodes of the lattice tree were then allocated based on the respective preferences of each class of preferred stock with the residual flowing to the common stock.

Probability-weighted Expected Return Method. Under the Probability-weighted Expected Return Method ("PWERM"), the value of Exicure OpCo's common stock is estimated based upon an analysis of values for Exicure OpCo assuming possible future events:

a) Initial public offering ("IPO")

b) Strategic merger or sale

c) Dissolution/No value to common

d) Private company

The per share value of the common stock using PWERM is based upon the probability-weighted present value of expected future equity values, under each of the possible future event scenarios, as well as the rights and preferences of each stock class.

Hybrid Method. As outlined in the Practice Aid, in certain circumstances, it may be appropriate to use a hybrid of certain allocation methodologies. In Exicure OpCo's circumstances, the per share values calculated under the Option Pricing Method and PWERM are weighted appropriately to arrive at a final fair market value per share value of Exicure OpCo's common stock.

Since March 31, 2015, Exicure OpCo's board has utilized the PWERM analysis to determine the fair value of Exicure OpCo's common stock in combination with the Option Pricing Method (see above) using a Hybrid Method. As discussed below, on those occasions the board utilized the Hybrid Method where they considered both the results from the Option Pricing Method and the results of PWERM analysis where particular consideration was given to the value of Exicure OpCo in the event of an IPO of Exicure OpCo's stock.

October 3, 2014. On October 3, 2014, Exicure OpCo's board determined that the fair value of Exicure OpCo's common stock was less than or equal to $0.64 per common share for all periods from the formation of Exicure OpCo until October 3, 2014. In its deliberation the board considered all of the qualitative and quantitative factors detailed above in the section captioned "Exicure OpCo common stock valuation."

As part of its determination the board considered four separate retrospective analyses of Exicure OpCo's common stock. Those analyses, each prepared by an independent third party appraiser in accordance with the Practice Aid, indicated a per share value for Exicure OpCo's common stock of $0.36 as of March 31, 2012, $0.42 as of July 31, 2013, and $0.70 as of June 30, 2014.

On October 3, 2014, the board determined the fair value of Exicure OpCo's common stock as of October 3, 2014 to be $0.64. In its deliberation the board considered all of the qualitative and quantitative factors detailed above in the section captioned "Exicure OpCo common stock valuation."

In determining the fair value of Exicure OpCo's common stock Exicure OpCo's board relied principally on the Back-solve Method for determining the enterprise value of Exicure OpCo and the Option Pricing Method for determining the fair value of Exicure OpCo's common stock. An important input into the application of the Back-solve Method was the June 2014 sale of Exicure OpCo's Series C preferred stock for net proceeds of $13.5 million at a per share price of $3.00.

April 28, 2015 and May 22, 2015. On April 28, 2015 and May 22, 2015, Exicure OpCo's board determined that the fair value of Exicure OpCo's common stock as of such dates was $1.03 per share. In its deliberation the board considered all of the qualitative and quantitative factors detailed above in the section captioned "Exicure OpCo common stock valuation."

In determining the fair value of Exicure OpCo's common stock, the board relied principally on the Back-solve Method for determining the enterprise value of Exicure OpCo. When determining the fair value of Exicure OpCo's common stock, the board, for the first time, considered both the probability of an IPO of Exicure OpCo's common stock in a PWERM analysis and the results provided by the Option Pricing Method. The fair value determined using the PWERM analysis was aggregated with the fair value determined by the Option Pricing Method using the Hybrid Method.

An important input into the application of the Back-solve Method was the February 2015 sale of Exicure OpCo's Series C preferred stock for aggregate proceeds of $4.6 million at a per share price of $3.00.

As part of its determination the board considered a contemporaneous valuation of Exicure OpCo's common stock prepared by an independent third party appraiser in accordance with the Practice Aid, which indicated a per share value for Exicure OpCo's common stock of $1.03 as of March 31, 2015. The board concluded in its deliberations that this valuation remained an important input into its deliberations since in its judgment no material change in the value of Exicure OpCo had occurred since March 31, 2015.

November 24, 2015. On November 24, 2015, Exicure OpCo's board determined that the fair value of Exicure OpCo's common stock as of such date was $1.97 per share. In its deliberation the board considered all of the qualitative and quantitative factors detailed above in the section captioned "Exicure OpCo common stock valuation."

In determining the fair value of Exicure OpCo's common stock, the board relied principally on the Back-solve Method for determining the enterprise value of Exicure OpCo. When determining the fair value of Exicure OpCo's common stock, the board considered both the probability of an IPO of Exicure OpCo's common stock in a PWERM analysis and the results provided by the Option Pricing Method. The fair value determined using the PWERM analysis was aggregated with the fair value determined by the Option Pricing Method using the Hybrid Method.

An important input into the application of the Back-solve Method was the October 2015 sale of Exicure OpCo's Series C preferred stock for aggregate proceeds of $15.0 million at a per share price of $3.00. As part of its determination the board considered a contemporaneous valuation of Exicure OpCo's common stock prepared by an independent third party appraiser in accordance with the Practice Aid, which indicated a per share value for Exicure OpCo's common stock of $1.97 as of September 30, 2015. The board concluded in its deliberations that this valuation remained an important input into its deliberations since in its judgment no material change in the value of Exicure OpCo had occurred since September 30, 2015.

In the board's judgment the increase in fair value from $0.64 at October 3, 2014 to $0.70 at December 31, 2014 to $1.03 at April 28, 2015 and May 22, 2015 to $1.97 at November 24, 2015 reasonably reflected both the scientific and clinical progress of Exicure OpCo's research programs and prospective therapeutic candidates as well as the possibility of an IPO.

May 11, 2016. On May 11, 2016, Exicure OpCo's board determined that the fair value of Exicure OpCo's common stock as of such date was $1.91 per share. In its deliberation the board considered all of the qualitative and quantitative factors detailed above in the section captioned "Exicure OpCo common stock valuation."

In determining the fair value of Exicure OpCo's common stock, the board relied principally on the Back-solve Method for determining the enterprise value of Exicure OpCo. When determining the fair value of Exicure OpCo's common stock, the board considered both the probability of an IPO of Exicure OpCo's common stock in a PWERM analysis and the results provided by the Option Pricing Method. The fair value determined using the PWERM analysis was aggregated with the fair value determined by the Option Pricing Method in the Hybrid Method. The board's analysis reflected that, based on then-existing market conditions, the IPO prospects for Exicure OpCo had diminished since the previous time the board determined fair value on November 24, 2015.

An important input into the application of the Back-solve Method was the October 2015 sale of Exicure OpCo's Series C preferred stock for aggregate proceeds of $15.0 million at a per share price of $3.00 and the January 2016 sale of Exicure OpCo's Series C preferred stock for aggregate proceeds of $0.4 million also at a per share price of $3.00. The Back-solve analysis also took into consideration changes in the market value of benchmark equity indices and the value created from cash spent on research and development ("R&D") activities. As part of its determination the board considered a contemporaneous valuation of Exicure OpCo's common stock prepared by an independent third party appraiser in accordance with the Practice Aid, which indicated a per share value for Exicure OpCo's common stock of $1.91 as of March 31, 2016. The board concluded in its deliberations that this valuation remained an important input into its deliberations since in its judgment no material change in the value of Exicure OpCo had occurred since March 31, 2016.

In the board's judgment the decrease in fair value from $1.97 at November 24, 2015 to $1.91 at May 11, 2016 reasonably reflected the diminished IPO prospects, based on then-existing market conditions, for Exicure OpCo

while also reflecting the scientific and clinical progress of Exicure OpCo's research programs and prospective therapeutic candidates.

August 10, 2016. On August 10, 2016, Exicure OpCo's board determined that the fair value of Exicure OpCo's common stock as of such date was $2.11 per share. In its deliberation the board considered all of the qualitative and quantitative factors detailed above in the section captioned "Exicure OpCo common stock valuation."

In determining the fair value of Exicure OpCo's common stock, the board relied principally on the Back-solve Method for determining the enterprise value of Exicure OpCo. When determining the fair value of Exicure OpCo's common stock, the board considered both the probability of an IPO of Exicure OpCo's common stock in a PWERM analysis and the results provided by the Option Pricing Method. The fair value determined using the PWERM analysis was aggregated with the fair value determined by the Option Pricing Method in the Hybrid Method. The board's analysis reflected that the IPO prospects for Exicure OpCo were generally consistent with those assumed in May 2016.

An important input into the application of the Back-solve Method was the October 2015 sale of Exicure OpCo's Series C preferred stock for aggregate proceeds of $15.0 million at a per share price of $3.00 and the January 2016 sale of Exicure OpCo's Series C preferred stock for aggregate proceeds of $0.4 million also at a per share price of $3.00. The Back-solve analysis also took into consideration changes in the market value of benchmark equity indices and the value created from cash spent on R&D activities. As part of its determination the board considered a contemporaneous valuation of Exicure OpCo's common stock prepared by an independent third party appraiser in accordance with the Practice Aid, which indicated a per share value for Exicure OpCo's common stock of $2.11 as of June 30, 2016. The board concluded in its deliberations that this valuation remained an important input into its deliberations since in its judgment no material change in the value of Exicure OpCo had occurred since June 30, 2016.

In the board's judgment the increase in fair value from $1.91 at May 11, 2016 to $2.11 at August 10, 2016 reasonably reflected the scientific and clinical progress of Exicure OpCo's research programs and prospective therapeutic candidates.

November 9, 2016. On November 9, 2016, Exicure OpCo's board determined that the fair value of Exicure OpCo's common stock as of such date was $2.42 per share. In its deliberation the board considered all of the qualitative and quantitative factors detailed above.

In determining the fair value of Exicure OpCo's common stock, the board relied principally on the Back-solve Method for determining the enterprise value of Exicure OpCo. When determining the fair value of Exicure OpCo's common stock, the board considered both the probability of an IPO of Exicure OpCo's common stock in a PWERM analysis and the results provided by the Option Pricing Method. The fair value determined using the PWERM analysis was aggregated with the fair value determined by the Option Pricing Method in the Hybrid Method. The board's analysis reflected that, based on then-existing market conditions, the IPO prospects for Exicure OpCo had improved as compared to the assumption at August 2016.

An important input into the application of the Back-solve Method was the October 2015 sale of Exicure OpCo's Series C preferred stock for aggregate proceeds of $15.0 million at a per share price of $3.00 and the January 2016 sale of Exicure OpCo's Series C preferred stock for aggregate proceeds of $0.4 million also at a per share price of $3.00. The Back-solve analysis also took into consideration changes in the market value of benchmark equity indices and the value created from cash spent on R&D activities. As part of its determination the board considered a contemporaneous valuation of Exicure OpCo's common stock prepared by an independent third party appraiser in accordance with the Practice Aid, which indicated a per share value for Exicure OpCo's common stock of $2.42 as of October 31, 2016. The board concluded in its deliberations that this valuation remained an important input into its deliberations since in its judgment no material change in the value of Exicure OpCo had occurred since October 31, 2016.

In the board's judgment the increase in fair value from $2.11 at August 10, 2016 to $2.42 at November 9, 2016 reasonably reflects both the scientific and clinical progress of Exicure OpCo's research programs and prospective

therapeutic candidates, as well as the completion of the Phase 1 trial for AST-005 and an understanding of the progress in negotiating the then pending Purdue Collaboration.

January 4, 2017. On January 4, 2017, Exicure OpCo's board determined that the fair value of Exicure OpCo's common stock as of such date was $4.21 per share. In its deliberation the board considered all of the qualitative and quantitative factors detailed above.

In determining the fair value of Exicure OpCo's common stock, the board relied principally on the Back-solve Method for determining the enterprise value of Exicure OpCo. When determining the fair value of Exicure OpCo's common stock, the board considered both the probability of an IPO of Exicure OpCo's common stock in a PWERM analysis and the results provided by the Option Pricing Method. The fair value determined using the PWERM analysis was aggregated with the fair value determined by the Option Pricing Method in the Hybrid Method. The board's analysis reflected that, based on then-existing market conditions, the IPO prospects for Exicure OpCo had improved as compared to the assumption at November 2016.

An important input into the application of the Back-solve Method was the October 2015 sale of Exicure OpCo's Series C preferred stock for aggregate proceeds of $15.0 million at a per share price of $3.00 and the January 2016 sale of Exicure OpCo's Series C preferred stock for aggregate proceeds of $0.4 million also at a per share price of $3.00. The Back-solve analysis also took into consideration changes in the market value of benchmark equity indices and the value created from cash spent on R&D activities. As part of its determination the board considered a contemporaneous valuation of Exicure OpCo's common stock prepared by an independent third party appraiser in accordance with the Practice Aid, which indicated a per share value for Exicure OpCo's common stock of $4.21 as of December 15, 2016. The board concluded in its deliberations that this valuation remained an important input into its deliberations since in its judgment no material change in the value of Exicure OpCo had occurred since December 15, 2016.

In the board's judgment the increase in fair value from $2.42 at November 9, 2016 to $4.21 at January 4, 2017 reasonably reflects both the scientific and clinical progress of Exicure OpCo's research programs and prospective therapeutic candidates, the execution of the Purdue Collaboration agreement on December 2, 2016, receipt of the upfront payment from Purdue, and the improved IPO prospects for Exicure OpCo.

Results of valuation models may vary. There are significant judgments and estimates inherent in the determination of these valuations. These judgments and estimates include assumptions regarding Exicure OpCo's future performance, including the successful enrollment and completion of Exicure OpCo's clinical studies as well as the determination of the appropriate valuation methods. If we had made different assumptions, our equity-based compensation expense could have been different. The foregoing valuation methodologies are not the only methodologies available and they will not be used after the Offering is complete. We cannot make assurances as to any particular valuation for Exicure OpCo's common stock. Accordingly, investors are cautioned not to place undue reliance on the foregoing valuation methodologies as an indicator of future stock prices.

Common stock warrant liability

Freestanding warrants related to shares that are redeemable, contingently redeemable, or for purchases of common stock that are not indexed to the Company's own stock are classified as a liability on the Company's balance sheet. The common stock warrants are recorded at fair value, estimated using the Black-Scholes option-pricing model, and marked to market at each balance sheet date with changes in the fair value of the liability recorded in other income (expense), net in the statements of operations.

A 10% change in the estimate of expected volatility at September 30, 2017 would increase or decrease the fair value of the common stock warrant liability in the amount of $18,000. A 10% change in the estimate of fair value of the common stock at September 30, 2017 would increase or decrease the fair value of the common stock warrant liability in the amount of $34,000.

Recent accounting pronouncements

In May 2014, the Financial Accounting Standards Board (“FASB”) issued Accounting Standards Update ("ASU") 2014-09 (ASC 606), Revenue from Contracts with Customers. This ASU, as amended by ASU 2015-14, affects any entity that either enters into contracts with customers to transfer goods and services or enters into contracts for the transfer of nonfinancial assets. ASU 2014-09 will replace most existing revenue recognition guidance in GAAP when it becomes effective. The standard’s core principle is that a company will recognize revenue when it transfers promised goods or services to customers in an amount that reflects the consideration to which the company expects to be entitled in exchange for those goods or services. In doing so, companies will need to use more judgment and make more estimates than under the currently effective guidance. These may include identifying performance obligations in the contract, estimating the amount of variable consideration to include in the transaction price and allocating the transaction price to each separate performance obligation. ASU 2014-09 is effective for Exicure in the first quarter of 2018 and early adoption is permitted beginning in the first quarter of 2017. The Company is currently evaluating the impact of this guidance on its financial statements.

In February 2016, FASB issued ASU 2016-02, Leases (Topic 842), which requires lessees to recognize right-of-use assets and lease liabilities on the balance sheet. ASU 2016-02 is to be applied using a modified retrospective approach at the beginning of the earliest comparative period in the financial statements. ASU 2016-02 will be effective for the Company beginning in the first quarter of 2019. Early adoption is permitted. The Company is currently evaluating the impact of adopting this standard on its financial statements.

In March 2016, the FASB issued ASU 2016-09, Improvements to Employee Share-Based Payment Accounting. ASU 2016-09 changes several aspects of the accounting for share-based payment transactions, including the income tax consequences, classification of awards as either equity or liabilities, and classification on the statement of cash flows. ASU 2016-09 requires all excess tax benefits and tax deficiencies to be recognized as income tax expense or benefit in the income statement and treated as discrete items in the reporting period. Further, excess tax benefits are required to be classified along with other income tax cash flows as an operating activity. The guidance was effective for the Company in the first quarter of 2017. Adoption of this guidance did not have a significant impact to the Company's financial statements.

In August 2016, the FASB issued ASU 2016-15, Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments. ASU 2016-15 addresses the classification of certain specific cash flow issues including debt prepayment or extinguishment costs, settlement of certain debt instruments, contingent consideration payments made after a business combination, proceeds from the settlement of certain insurance claims and distributions received from equity method investees. ASU 2016-15 is effective for the Company in the first quarter of 2018 and early adoption is permitted. An entity that elects early adoption must adopt all of the amendments in the same period. The Company is currently evaluating the impact of this guidance on its statement of cash flows.

In May 2017, the FASB issued ASU 2017-09, Compensation - Stock Compensation (Topic 718): Scope of Modification Accounting. ASU 2017-09 clarifies when changes to the terms or conditions of a share-based payment award must be accounted for as modifications. Under the new guidance, modification accounting is required only if the fair value, the vesting conditions, or the classification of the award changes as a result of the change in terms or conditions. ASU 2017-09 will be applied prospectively to awards modified on or after the adoption date. ASU 2017-09 is effective for the Company for fiscal years beginning after December 15, 2017, and interim periods within those fiscal years. Early adoption is permitted. The Company is currently evaluating the impact of this guidance on its financial statements.

Components of Statements of Operations

Revenue

We have earned all of our revenue through September 30, 2017 through our research collaboration, license, and option agreement with Purdue or as a primary contractor or as a subcontractor on government grants. We do not intend for government grants to be a principal commercial or strategic focus, but will evaluate opportunities when consistent with our strategic priorities. We have not generated any commercial product revenue and do not expect to generate any product revenue for the foreseeable future.

In the future, we may generate revenue from partnership activities including a combination of research and development payments, license fees and other upfront payments, milestone payments, product sales and royalties, and reimbursement of certain research and development expenses, in connection with the Purdue Collaboration or any future collaborations and licenses. We expect that any such revenue we generate will fluctuate in future periods as a result of the timing of achievement, if at all, of preclinical, clinical, regulatory and commercialization milestones, the timing and amount of any payments to us relating to such milestones and the extent to which any of our therapeutic candidates are approved and successfully commercialized by us or potential development partners. If we, or any potential development partner fails to develop therapeutic candidates in a timely manner or obtain regulatory approval for them, our ability to generate future revenue, and our results of operations and financial position, would be materially and adversely affected.

Research and development expense

Research and development expense consists of costs associated with our research activities, including discovery and development of our SNAs, and their therapeutic applications. Our research and development expenses include:

- direct research and development expenses incurred under arrangements with third parties, such as contract research organizations, contract manufacturing organizations, and consultants;
- laboratory materials and supplies;
- costs of maintaining our intellectual property portfolio, including license fees, sublicense fees, patent maintenance and other similar fees;
- employee-related expenses, including salaries, bonuses, benefits and equity-based compensation expense; and
- facilities, depreciation and other allocated expenses, which include direct and allocated expenses for rent and maintenance of facilities, depreciation of leasehold improvements and equipment and laboratory and other supplies.

We expense research and development costs as they are incurred. A significant portion of our research and development costs are not tracked by project as they benefit multiple projects or our technology.

We expect our research and development expenses to increase for the foreseeable future as we advance our therapeutic candidates through preclinical studies and clinical trials. The process of conducting preclinical studies and clinical trials necessary to obtain regulatory approval is costly and time-consuming. We or future development partners may never succeed in obtaining marketing approval for any of our therapeutic candidates. The probability of success for each therapeutic candidate may be affected by numerous factors, including preclinical data, clinical data, competition, manufacturing capability and commercial viability.

All of our research and development programs are at an early stage and successful development of future therapeutic candidates from these programs is highly uncertain and may not result in approved products. Completion dates and completion costs can vary significantly for each future therapeutic candidate and are difficult to predict. We anticipate we will make determinations as to which therapeutic candidates to pursue and how much funding to direct to each therapeutic candidate on an ongoing basis in response to our ability to maintain or enter into development partnership with respect to each therapeutic candidate, the scientific and clinical success of each therapeutic candidate as well as ongoing assessments as to the commercial potential of therapeutic candidates. We will need to raise additional capital and may seek collaborations in the future in order to advance our various therapeutic candidates. Additional private or public financings may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a material adverse effect on our financial condition and our ability to pursue our business strategy.

General and administrative expense

General and administrative expense consists primarily of salaries and related benefits, including equity-based compensation, related to our executive, finance, legal, business development and support functions. Other general

and administrative expenses include travel expenses, professional fees for auditing, tax and legal services and allocated facility-related costs not otherwise included in research and development expenses.

We expect that general and administrative expenses will increase in the future as we expand our operating activities and incur additional costs associated with being a publicly-traded company. These increases will likely include legal, accounting and filing fees, directors' and officers' liability insurance premiums and fees associated with investor relations.

Interest expense

Interest expense consists of interest expense pursuant to the loan and security agreement with Hercules that we closed on February 17, 2016 with an initial advance of $6.0 million.

Other income (loss), net

Other income (loss), net consists of interest income earned on our cash and cash equivalents, fair value adjustments of our preferred and common stock warrant liabilities, and gains and losses on foreign currency transactions.

Results of Operations

Comparison of the Three Months Ended September 30, 2017 and 2016

The following table summarizes the results of our operations for the three months ended September 30, 2017 and 2016 (in thousands, except percentages):

	Three Months Ended September 30,			
	2017	2016	Change	
Revenue:				
Collaboration revenue	$ 2,497	$ —	$ 2,497	n/m
Grant income	—	—	—	n/m
Total revenue	2,497	—	2,497	n/m
Operating expenses:				
Research and development expense	3,502	2,413	1,089	45 %
General and administrative expense	1,270	849	421	50 %
Total operating expenses	4,772	3,262	1,510	46 %
Operating loss	(2,275)	(3,262)	987	(30)%
Other income (expense), net:				
Interest expense	(201)	(208)	7	(3)%
Other income (loss), net	163	(56)	219	n/m
Total other income (loss), net	(38)	(264)	226	(86)%
Net loss	$ (2,313)	$ (3,526)	$ 1,213	(34)%

Revenue

(dollars in thousands)	Three Months Ended September 30, 2017	2016	Change	
Collaboration revenue	$ 2,497	$ —	$ 2,497	n/m
Grant income	—	—	—	n/m
Total revenue	$ 2,497	$ —	$ 2,497	n/m

We recognized collaboration revenue of $2.5 million for the three months ended September 30, 2017. In connection with the Purdue Collaboration, we received a non-refundable development fee of $10.0 million in December 2016 which was deferred and is recognized as collaboration revenue over the period in which the revenue is earned. The collaboration revenue of $2.5 million during the three months ended September 30, 2017 included $0.4 million of research and development activities that is reimbursable by Purdue and is presented on a gross basis in the accompanying consolidated statement of operations. We did not have any active grants as of September 30, 2017 and 2016, and therefore did not recognize any grant income during the three months ended September 30, 2017 and 2016.

We do not intend for government grants to be a principal commercial or strategic focus, but will evaluate opportunities when consistent with our strategic priorities. We do not expect to generate any product revenue for the foreseeable future. However, future revenue may include amounts attributable to partnership activities including, a combination of research and development payments, license fees and other upfront payments, milestone payments, product sales and royalties, and reimbursement of certain research and development expenses, in connection with the Purdue Collaboration or any future collaboration and licenses.

Research and development expense

The following table summarizes our research and development expenses incurred during the periods indicated:

(dollars in thousands)	Three Months Ended September 30, 2017	2016	Change	
Clinical development programs expense	$ 1,839	$ 801	$ 1,038	130 %
Platform and discovery-related expense	821	841	(20)	(2)%
Employee-related expense	618	555	63	11 %
Facilities, depreciation, and other expenses	224	216	8	4 %
Total research and development expense	$ 3,502	$ 2,413	$ 1,089	45 %
Full time employees	17	15	2	

Research and development expense was $3.5 million for the three months ended September 30, 2017 and $2.4 million for the three months ended September 30, 2016, an increase of $1.1 million, or 45%. Included in clinical development expense for the three months ended September 30, 2017 was approximately $0.4 million of expense that is reimbursed by Purdue (included in revenue) related to the Phase 1b trial of AST-005. The increase in research and development expense of $1.1 million was primarily due to a net increase in costs related to our clinical development programs of approximately $1.0 million. This net increase was mostly due to higher costs for preparation for the Phase 1 clinical trial of AST-008, in addition to higher costs related to the preparation for the Phase 1 clinical trial of XCUR17 and higher costs related to the Phase 1b clinical trial of AST-005.

We expect our research and development expenses to increase in 2018 as we continue spending on our therapeutic development programs, further develop our SNA technology platform and broaden our pipeline of SNA-based candidates.

General and administrative expense

(dollars in thousands)	Three Months Ended September 30, 2017	2016	Change	
General and administrative expense	$ 1,270	$ 849	$ 421	50%
Full time employees	7	6	1	

General and administrative expense was $1.3 million for the three months ended September 30, 2017 and $0.8 million for the three months ended September 30, 2016, an increase of $0.4 million, or 50%. This increase is primarily due to higher equity-based compensation expense of $0.2 million, mostly related to stock options granted in January 2017 and expense of $0.2 million related to the termination of a contract with a placement agent that did not participate in the Offering.

We expect that general and administrative expenses will increase in the future as we expand our operating activities and incur additional costs associated with being a publicly-traded company. These increases will likely include legal, accounting and filing fees, directors' and officers' liability insurance premiums and fees associated with investor relations.

Interest expense

Interest expense consists of interest expense pursuant to the loan and security agreement with Hercules that we closed on February 17, 2016 with an initial advance of $6.0 million.

Other income (loss), net

Other income (loss), net consists of interest income earned on our cash and cash equivalents, fair value adjustments of our preferred and common stock warrant liabilities, and gains and losses on foreign currency transactions. Other income, net of $0.2 million for the three months ended September 30, 2017 included a gain of $0.2 million in connection with the fair value adjustment of the liability for the preferred stock warrants that terminated in connection with the Merger.

Comparison of the Nine Months Ended September 30, 2017 and 2016

The following table summarizes the results of our operations for the nine months ended September 30, 2017 and 2016 (in thousands, except percentages):

	Nine Months Ended September 30,			
	2017	2016	Change	
Revenue:				
Collaboration revenue	$ 7,624	$ —	$ 7,624	n/m
Grant income	—	346	(346)	n/m
Total revenue	7,624	346	7,278	n/m
Operating expenses:				
Research and development expense	11,279	8,329	2,950	35 %
General and administrative expense	4,806	2,736	2,070	76 %
Total operating expenses	16,085	11,065	5,020	45 %
Operating loss	(8,461)	(10,719)	2,258	(21)%
Other income (expense), net:				
Interest expense	(616)	(516)	(100)	19 %
Other income (loss), net	140	(64)	204	n/m
Total other income (loss), net	(476)	(580)	104	(18)%
Net loss	$ (8,937)	$ (11,299)	$ 2,362	(21)%

Revenue

	Nine Months Ended September 30,			
(dollars in thousands)	2017	2016	Change	
Collaboration revenue	$ 7,624	$ —	$ 7,624	n/m
Grant income	—	346	(346)	n/m
Total revenue	$ 7,624	$ 346	$ 7,278	n/m

We recognized revenue of $7.6 million for the nine months ended September 30, 2017. In connection with the Purdue Collaboration, we received a non-refundable development fee of $10.0 million in December 2016 which was deferred and is recognized as collaboration revenue over the period in which the revenue is earned. We recognized $7.6 million of collaboration revenue during the nine months ended September 30, 2017, which included $1.4 million of research and development activities that is reimbursable by Purdue and is presented on a gross basis in the accompanying consolidated statement of operations. The grant income revenue of $0.3 million recognized in the nine months ended September 30, 2016 related to our performance as a primary contractor or as a subcontractor on government grants that concluded in early 2016. We did not have any active grants as of September 30, 2017 and 2016.

We do not intend for government grants to be a principal commercial or strategic focus, but will evaluate opportunities when consistent with our strategic priorities. We do not expect to generate any product revenue for the foreseeable future. However, future revenue may include amounts attributable to partnership activities including, a combination of research and development payments, license fees and other upfront payments, milestone payments, product sales and royalties, and reimbursement of certain research and development expenses, in connection with the Purdue Collaboration or any future collaboration and licenses.

Research and development expense

The following table summarizes our research and development expenses incurred during the periods indicated:

	Nine Months Ended September 30,			
(dollars in thousands)	2017	2016	Change	
Clinical development programs expense	$ 6,221	$ 3,326	$ 2,895	87 %
Platform and discovery-related expense	2,494	2,702	(208)	(8)%
Employee-related expense	1,916	1,702	214	13 %
Facilities, depreciation, and other expenses	648	599	49	8 %
Total research and development expense	$ 11,279	$ 8,329	$ 2,950	35 %
Full time employees	17	15	2	

Research and development expense was $11.3 million for the nine months ended September 30, 2017 and $8.3 million for the nine months ended September 30, 2016, an increase of $3.0 million, or 35%. Included in clinical development expense for the nine months ended September 30, 2017 was approximately $1.4 million of expense that is reimbursed by Purdue (included in revenue) related to the Phase 1b trial of AST-005. The increase in research and development expense of $3.0 million was primarily due to a net increase in costs related to our clinical development programs of $2.9 million. This net increase was mostly due to higher costs for preparation for the Phase 1 clinical trials of both AST-008 and XCUR17. Higher employee-related expense of $0.2 million, mostly the result of an increase in headcount and salary increases, essentially offset lower platform and discovery-related expense of $0.2 million, which reflected lower costs related to contract research organizations and higher costs to maintain our intellectual property portfolio.

We expect our research and development expenses to increase in 2018 as we continue spending on our clinical development programs, further develop our SNA technology platform and broaden our pipeline of SNA-based candidates.

General and administrative expense

	Nine Months Ended September 30,			
(dollars in thousands)	2017	2016	Change	
General and administrative expense	$ 4,806	$ 2,736	$ 2,070	76%
Full time employees	7	6	1	

General and administrative expense was $4.8 million for the nine months ended September 30, 2017 and $2.7 million for the nine months ended September 30, 2016, an increase of $2.1 million, or 76%. This increase is primarily due to higher costs for legal and accounting services of approximately $1.1 million, which were incurred in support of a potential public offering that was abandoned during the second quarter of 2017. Higher equity-based compensation expense of $0.6 million, mostly related to stock options granted in January 2017, and expense of $0.2 million related to the termination of a contract with a placement agent that did not participate in the Offering also contributed to the increase in general and administrative expense in the nine months ended September 30, 2017.

We expect that general and administrative expenses will increase in the future as we expand our operating activities and incur additional costs associated with being a publicly-traded company. These increases will likely include legal, accounting and filing fees, directors' and officers' liability insurance premiums and fees associated with investor relations.

Interest expense

Interest expense consists of interest expense pursuant to the loan and security agreement with Hercules that we closed on February 17, 2016 with an initial advance of $6.0 million.

Other income (loss), net

Other income (loss), net consists of interest income earned on our cash and cash equivalents, fair value adjustments of our preferred and common stock warrant liabilities, and gains and losses on foreign currency transactions. Other income, net of $0.1 million for the nine months ended September 30, 2017 included a gain of $0.2 million in connection with the fair value adjustment of the liability for the preferred stock warrants that terminated in connection with the Merger.

Liquidity and Capital Resources

Overview

To date we have primarily funded our operations through private placements of equity securities, the Purdue Collaboration, a debt financing, and grants from governmental agencies. Through September 30, 2017, we have received approximately $84.1 million in aggregate gross proceeds from these transactions, including $42.8 million in aggregate gross proceeds from private placement offerings of preferred stock, $20.3 million gross proceeds from the initial closing of the Offering on September 26, 2017, an upfront payment of $10.0 million in connection with the Purdue Collaboration, $6.0 million in debt financing, and an aggregate of $5.0 million from grants awarded by governmental agencies.

On October 27, 2017 and November 2, 2017, we sold a total of 3,736,836 shares of the Company's common stock, par value $0.0001 per share in subsequent closings of the Offering, resulting in approximately $11.2 million in gross proceeds to the Company.

Since our inception, we have not generated any product revenue and have incurred recurring net losses. Our Company is not profitable, and we cannot provide any assurance that we will ever be profitable. As of September 30, 2017, we have an accumulated deficit of $50.4 million. We believe that our cash and cash equivalents as of September 30, 2017 of $22.9 million, when combined with the aggregate cash raised on October 27, 2017 and November 2, 2017 of approximately $11.2 million for the sale of common stock in the Offering, but excluding any potential proceeds from option exercises, is sufficient to fund the Company into 2019. See "—Funding Requirements" below for additional information on our future capital needs.

Cash Flows

The following table shows a summary of our cash flows for the nine months ended September 30, 2017 and 2016:

(in thousands)	Nine Months Ended September 30,	
	2017	**2016**
	(unaudited)	(unaudited)
Net cash used in operating activities	$ (14,447)	$ (10,677)
Net cash used in investing activities	(726)	(315)
Net cash provided by financing activities	18,486	6,329
Net increase (decrease) in cash and cash equivalents	$ 3,313	$ (4,663)

Operating activities

Net cash used in operating activities was $14.4 million and $10.7 million for the nine months ended September 30, 2017 and 2016, respectively. The increase in cash used in operating activities of $3.8 million was primarily due to the cash impact of higher research and development expenses in the nine months ended September 30, 2017, the payment of $1.5 million in connection with the Northwestern University License Agreements, the payment for legal and accounting services of approximately $1.1 million, which were incurred in support of a potential public offering that was abandoned during the second quarter of 2017, as well as the prepayment of directors and officers liability insurance.

Investing activities

Net cash used in investing activities was $0.7 million and $0.3 million for the nine months ended September 30, 2017 and 2016, respectively. Cash used in investing activities for each of the above-mentioned periods was primarily due to the purchase of scientific equipment.

Financing activities

Net cash provided by financing activities of $18.5 million during the nine months ended September 30, 2017 is primarily due to the sale of common stock in the Offering on September 26, 2017. Gross proceeds from the Offering of $20.3 million, less financing costs of $1.3 million, were partially offset by the repayment of debt of $0.6 million. Net cash provided by financing activities of $6.3 million during the nine months ended September 30, 2016 is mostly due to net proceeds of $5.9 million from our February 2016 secured debt financing with Hercules and $0.4 million from our January 2016 Series C preferred stock offering.

Hercules Loan and Security Agreement

On February 17, 2016, we entered into a loan and security agreement with Hercules. The loan agreement provided for funding in an aggregate principal amount of up to $10.0 million in two separate tranches. The first tranche was funded on February 17, 2016 in the amount of $6.0 million. A second tranche of $4.0 million was available provided that we met certain milestones on or before December 31, 2016. We did not meet these milestones and, therefore, we did not draw the second tranche, the availability of which expired on December 31, 2016. The principal balance of the term loan under the Hercules loan facility bears interest at a floating per annum interest rate (based on a year consisting of 360 days) equal to the greater of either (i) 9.95% or (ii) the sum of (a) 9.95% plus (b) the prime rate (as reported in The Wall Street Journal) minus 3.50%. We were required to make interest-only payments through June 2017. Commencing on July 1, 2017, the loan began amortizing in equal monthly installments of principal and interest in an amount sufficient to fully amortize the outstanding principal balance of the loan over the remaining scheduled monthly payments due prior to the maturity date on September 1, 2019. Any remaining obligations under the loan agreement and other loan documents (other than the warrant described below) are due and payable on the maturity date. On the earliest to occur of the maturity date, the date we prepay the term loan in full or the date the loan otherwise becomes due and payable, we must pay the lender under the agreement an additional charge equal to 3.85% of the total amounts funded under the loan agreement. In addition, if we prepaid the term loan on or prior to February 1, 2017, we would be required to pay a prepayment charge equal to 3% of the amount being prepaid, if we prepay the term loan after February 1, 2017 but on or prior to February 1, 2018, we must pay a prepayment charge equal to 2% of the amount being prepaid, and if the we prepay the term loan after February 1, 2018, we must pay a prepayment charge of 1% of the amount being prepaid. The loan agreement was amended on October 10, 2016 to revise the language granting Hercules a contingent security interest in certain of our assets.

The term loan under the Hercules loan facility is secured by substantially all of our assets, other than intellectual property, which is the subject of a negative pledge. Under the loan agreement, we are subject to certain customary covenants that limit or restrict our ability to, among other things, incur additional indebtedness, grant any security interests, pay cash dividends, repurchase our common stock, make loans, or enter into certain transactions without Hercules' prior consent.

Under the loan agreement, Hercules or its affiliates have a right to participate in a single subsequent unregistered financing by us in an amount of up $1.0 million on the same terms, conditions and pricing afforded to others participating in such financing. Hercules has not yet exercised this right to participate.

In connection with our financing with Hercules, we issued Hercules a warrant to purchase up to 80,000 shares of our Series C Preferred Stock at an exercise price of $3.00 per share, plus an additional number of shares in the event that the second tranche of funding had been drawn before it expired on December 31, 2016. The warrant terminated at the closing of the Merger.

Funding Requirements

We expect that our primary uses of capital will continue to be third-party clinical research and development services, compensation and related expenses, laboratory and related supplies, legal and other regulatory expenses and general overhead costs. We believe that our existing cash and cash equivalents as of September 30, 2017, when combined with the aggregate cash raised on October 27, 2017 and November 2, 2017 of approximately $11.2 million for the sale of common stock in the Offering, but excluding any potential proceeds from option exercises, is sufficient to meet our anticipated cash requirements into 2019. However, we may require additional capital for the further development of our existing therapeutic candidates and may also need to raise additional funds sooner to pursue other development activities related to additional therapeutic candidates. We believe that we will be able to obtain additional working capital through equity financings, partnerships and licensing, or other arrangements to fund our current operating plans into 2019, which we believe will allow us to execute on the strategy and pipeline development as described in this Quarterly Report on Form 10-Q. To the extent that we raise additional capital through future equity financings, the ownership interest of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our existing common stockholders. If we raise additional funds through the issuance of debt securities, these securities could contain covenants that would restrict our operations. We cannot assure you that such additional financing, if available, can be obtained on terms acceptable to us. If we are unable to obtain such additional financing, we would need to reevaluate our future operating plans.

Our forecast of the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement that involves risks and uncertainties, and actual results could vary materially as a result of a number of factors. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we currently expect. Our future capital requirements are difficult to forecast and will depend on many factors, including:

- the terms and timing of any other collaboration, licensing and other arrangements that we may establish;
- the initiation, progress, timing and completion of preclinical studies and clinical trials for our potential therapeutic candidates;
- the number and characteristics of therapeutic candidates that we pursue;
- the progress, costs and results of our preclinical studies and clinical trials;
- the outcome, timing and cost of regulatory approvals;
- delays that may be caused by changing regulatory requirements;
- the cost and timing of hiring new employees to support our continued growth;
- unknown legal, administrative, regulatory, accounting, and information technology costs as well as additional costs associated with operating as a public company;
- the costs involved in filing and prosecuting patent applications and enforcing and defending patent claims;
- the costs of filing and prosecuting intellectual property rights and enforcing and defending any intellectual property-related claims;

- the costs and timing of procuring clinical and commercial supplies of our therapeutic candidates;
- the extent to which we acquire or in-license other therapeutic candidates and technologies; and
- the extent to which we acquire or invest in other businesses, therapeutic candidates or technologies.

Please see the section titled "Risk Factors" elsewhere in this Quarterly Report on Form 10-Q for additional risks associated with our substantial capital requirements.

Until such time, if ever, we generate product revenue, we expect to finance our cash needs through a combination of public or private equity offerings, debt financings and research collaboration and license agreements. We may be unable to raise capital or enter into such other arrangements when needed or on favorable terms or at all. Our failure to raise capital or enter into such other arrangements as and when needed would have a negative impact on our financial condition and our ability to develop our therapeutic candidates.

Contractual Obligations and Commitments

There have been no material changes outside the ordinary course of business during the period covered by this Quarterly Report on Form 10-Q from the contractual obligations and commitments as of December 31, 2016 described in our Form 8-K.

Off-balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

JOBS Act

In April 2012, the Jumpstart Our Business Startups Act of 2012 (the "JOBS Act") was enacted by the federal government. Section 107 of the JOBS Act provides that an emerging growth company can take advantage of the extended transition period for complying with new or revised accounting standards. Thus, an emerging growth company can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this extended transition period and, as a result, we will adopt new or revised accounting standards on the relevant dates on which adoption of such standards is required for other public companies.

In addition, as an emerging growth company, we will not be required to provide an auditor's attestation report on our internal control over financial reporting in future annual reports on Form 10-K as otherwise required by Section 404(b) of the Sarbanes-Oxley Act.

Item 3. Quantitative and Qualitative Disclosures about Market Risk.

The primary objectives of our investment activities are to ensure liquidity and to preserve principal while at the same time maximizing the income we receive from our marketable securities without significantly increasing risk. Some of the securities that we invest in may have market risk related to changes in interest rates. As of September 30, 2017 and December 31, 2016, we had cash equivalents of $22.9 million and $19.6 million, respectively, consisting of interest-bearing money market accounts. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. Due to the short-term maturities of our cash equivalents and the low risk profile of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our cash equivalents. To minimize the risk in the future, we intend to maintain our portfolio of cash equivalents and short-term investments in a variety of securities, including commercial paper, money market funds, government and non-government debt securities and corporate obligations.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2017. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the "Exchange Act"), means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to its management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of September 30, 2017, our principal executive officer and principal financial officer concluded that, as of such date, our disclosure controls and procedures were effective.

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting identified in connection with the evaluation required by Rule 13a-15(d) and 15d-15(d) of the Exchange Act that occurred during the fiscal quarter ended September 30, 2017 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II – OTHER INFORMATION

Item 1. Legal Proceedings.

From time to time, we may be subject to legal proceedings. We are not currently a party to or aware of any proceedings that we believe will have, individually or in the aggregate, a material adverse effect on our business, financial condition or results of operations.

Item 1A. Risk Factors.

Investing in our common stock involves a high degree of risk. In addition to the other information set forth in this Quarterly Report on Form 10-Q, you should carefully consider the risk factors discussed below when considering an investment in our common stock. If any of the following risks occur, our business, financial condition, results of operations and prospects could be materially and adversely affected. In that case, the market price of our common stock could decline and you could lose some or all of your investment. Additional risks and uncertainties not presently known to us or that we currently deem immaterial also may impair our business operations.

Risks Related to Our Business

We are a clinical-stage biotechnology company with a history of losses. We expect to continue to incur significant losses for the foreseeable future and may never achieve or maintain profitability, which could result in a decline in the market value of our common stock.

We are a biotechnology company developing gene regulatory and immuno-oncology therapeutics based on our proprietary SNA technology. We have a limited operating history. Since our inception in June 2011, we have devoted our resources to the development of SNA technology. We have had significant operating losses since our inception. As of September 30, 2017, we have generated an accumulated deficit of $50.4 million. For the nine months ended September 30, 2017 and 2016, our net loss was $8.9 million and $11.3 million, respectively. Substantially all of our losses have resulted from expenses incurred in connection with our research programs and from general and administrative costs associated with our operations. Our technology and therapeutic candidates are in early stages of development, and we are subject to the risks of failure inherent in the development of therapeutic candidates based on novel technologies.

We have not generated, and do not expect to generate, any product revenue for the foreseeable future, and we expect to continue to incur significant operating losses for the foreseeable future due to the cost of research and development, preclinical studies, clinical trials, and the regulatory approval process for therapeutic candidates. The amount of future losses is uncertain. Our ability to achieve profitability, if ever, will depend on, among other things, us, or any current or future collaborators, successfully developing therapeutic candidates, obtaining regulatory approvals to market and commercialize therapeutic candidates, manufacturing any approved products on commercially reasonable terms, establishing a sales and marketing organization or suitable third party alternatives for any approved product and raising sufficient funds to finance business activities. If we, or any current or future collaborators, are unable to develop and commercialize one or more of our therapeutic candidates or if sales revenue from any therapeutic candidate that receives approval is insufficient, we will not achieve profitability, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Our approach to the discovery and development of innovative therapeutic treatments based on our technology is unproven and may not result in marketable products.

We plan to develop a pipeline of therapeutic candidates using our proprietary SNAs as therapeutic agents. We believe that therapeutic candidates identified with our therapeutic discovery technology may offer an improved therapeutic approach to small molecules and antibodies, as well as several advantages over linear oligonucleotide-based therapeutics. However, the scientific research that forms the basis of our efforts to develop therapeutic candidates based on our SNAs and the identification and optimization of SNAs is relatively new. Further, the

scientific evidence to support the feasibility of developing therapeutic treatments based on SNAs is both preliminary and limited.

Therapeutic candidates based on SNA technology have not been extensively tested in humans, and a number of clinical trials conducted by other companies using oligonucleotide technologies have not been successful. We may discover that the SNAs do not possess certain properties required for therapeutic treatment to be effective, such as the ability to remain stable in the human body for the period of time required for the therapeutic candidate to reach the target tissue or the ability to cross the cell membrane and enter into cells within the target tissue for effective delivery. We currently have only limited data, and no conclusive evidence, to suggest that we can introduce these necessary drug-like properties into SNAs. We may spend substantial funds attempting to introduce these properties and may never succeed in doing so. In addition, therapeutic candidates based on SNAs may demonstrate different chemical and pharmacological properties in patients than they do in laboratory studies. Even if therapeutic candidates have successful results in animal studies, they may not demonstrate the same chemical and pharmacological properties in humans and may interact with human biological systems in unforeseen, ineffective or harmful ways. As a result, we may never succeed in developing a marketable therapeutic, we may not become profitable and the value of our common stock will decline.

Further, the Food and Drug Administration ("FDA") has limited experience with SNA-based therapeutics. No regulatory authority has granted approval to any person or entity, including us, to market and commercialize therapeutics using SNAs, which may increase the complexity, uncertainty and length of the regulatory approval process for our therapeutic candidates. We and any current or future collaborators may never receive approval to market and commercialize any therapeutic candidate. Even if we or a future collaborator obtain regulatory approval, the approval may be for disease indications or patient populations that are not as broad as we intended or desired or may require labeling that includes significant use or distribution restrictions or safety warnings. We or a future collaborator may be required to perform additional or unanticipated clinical trials to obtain approval or be subject to post-marketing testing requirements to maintain regulatory approval. If our SNA technology proves to be ineffective, unsafe or commercially unviable, our technology and pipeline would have little, if any, value, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Our therapeutic candidates are in early stages of development and may fail in development or suffer delays that materially and adversely affect their commercial viability.

We have no therapeutics on the market and all of our therapeutic candidates are in early stages of development. Our ability to achieve and sustain profitability depends on obtaining regulatory approvals, including an institutional review board ("IRB") approval to conduct clinical trials at particular sites for, and successfully commercializing, our therapeutic candidates, either alone or with third parties. Before obtaining regulatory approval for the commercial distribution of our therapeutic candidates, we or an existing or a future collaborator must conduct extensive preclinical studies and clinical trials to demonstrate the safety and efficacy in humans of our therapeutic candidates. Preclinical studies and clinical trials are expensive, difficult to design and implement, can take many years to complete and are uncertain as to outcome. The start or end of a clinical trial is often delayed or halted due to changing regulatory requirements, manufacturing challenges, required clinical trial administrative actions, slower than anticipated patient enrollment, changing standards of care, availability or prevalence of use of a comparative therapeutic or required prior therapy, clinical outcomes or financial constraints. For instance, delays or difficulties in patient enrollment or difficulties in retaining trial participants can result in increased costs, longer development times or termination of a clinical trial. Clinical trials of a new therapeutic candidate require the enrollment of a sufficient number of patients, including patients who are suffering from the disease the therapeutic candidate is intended to treat and who meet other eligibility criteria. Rates of patient enrollment are affected by many factors, including the size of the patient population, the eligibility criteria for the clinical trial, the age and condition of the patients, the stage and severity of disease, the nature of the protocol, the proximity of patients to clinical sites and the availability of effective treatments for the relevant disease.

A therapeutic candidate can unexpectedly fail at any stage of preclinical and clinical development. In our completed Phase 1 trial, AST-005 did not show an antipsoriatic effect. Although we believe this result was due to the short length of this clinical trial, there is no guarantee that AST-005 will show an antipsoriatic effect in future clinical trials of longer duration. The historical failure rate for therapeutic candidates is high due to scientific

feasibility, safety, efficacy, changing standards of medical care and other variables. The results from preclinical studies or early clinical trials of a therapeutic candidate may not predict the results that will be obtained in later phase clinical trials of the therapeutic candidate. We, the FDA, an IRB, an independent ethics committee, or other applicable regulatory authorities may suspend clinical trials of a therapeutic candidate at any time for various reasons, including a finding that subjects participating in such trials are being exposed to unreasonable and significant risk of illness or injury. Similarly, an IRB or ethics committee may suspend a clinical trial at a particular trial site. We may not have the financial resources to continue development of, or to enter into collaborations for, a therapeutic candidate if we experience any problems or other unforeseen events that delay or prevent regulatory approval of, or our ability to commercialize, therapeutic candidates, including:

- negative or inconclusive results from our clinical trials or the clinical trials of others for therapeutic candidates similar to ours, leading to a decision or requirement to conduct additional preclinical testing or clinical trials or abandon a program;
- therapeutic-related side effects experienced by participants in our clinical trials or by individuals using therapeutics similar to our therapeutic candidates;
- delays in submitting INDs or CTAs, or comparable foreign applications or delays or failure in obtaining the necessary approvals from regulators or IRBs to commence a clinical trial, or a suspension or termination of a clinical trial once commenced;
- conditions imposed by the FDA or comparable foreign authorities, such as the European Medicines Agency ("EMA"), or European Union national competent authorities, regarding the scope or design of our clinical trials;
- delays in enrolling research subjects in clinical trials;
- inadequate supply or quality of therapeutic candidate components or materials or other supplies necessary for the conduct of our clinical trials;
- high drop-out rates of research subjects;
- greater than anticipated clinical trial costs;
- poor effectiveness of our therapeutic candidates during clinical trials;
- unfavorable FDA or other regulatory agency inspection and review of a clinical trial site;
- failure of our third party contractors or investigators to comply with regulatory requirements or otherwise meet their contractual obligations in a timely manner, or at all;
- delays and changes in regulatory requirements, policy and guidelines, including the imposition of additional regulatory oversight around clinical testing generally or with respect to our technology in particular; or
- varying interpretations of data by the FDA and similar foreign regulatory agencies.

Product development involves a lengthy and expensive process with an uncertain outcome, and results of earlier preclinical studies and clinical trials may not be predictive of future clinical trial results.

Clinical testing is expensive and generally takes many years to complete, and the outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. The results of preclinical studies and early clinical trials of our therapeutic candidates may not be predictive of the result of larger, later-stage controlled clinical trials. Therapeutic candidates that have shown promising results in early stage clinical trials may still suffer significant setbacks in subsequent clinical trials. We will have to conduct trials in our proposed indications to verify the results obtained to date and to support any regulatory submissions for further clinical development. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles despite promising results in earlier, smaller clinical trials. Moreover, clinical data is often susceptible to varying interpretations and analyses. We do not know whether Phase 1, Phase 2, Phase 3, or other clinical trials we may conduct will demonstrate consistent or adequate efficacy and safety with respect to the proposed indication for use sufficient to receive regulatory approval or market our therapeutic candidates.

We will need substantial additional funds to advance the development of our therapeutic candidates, and we cannot guarantee that we will have sufficient funds available in the future to develop and commercialize our current or future therapeutic candidates.

If our existing therapeutic candidates or our future therapeutic candidates enter and advance through preclinical studies and clinical trials, we will need substantial additional funds to expand our development, regulatory, manufacturing, marketing, and sales capabilities or contract with other organizations to provide these capabilities for us. We have used substantial funds to develop our therapeutic candidates and will require significant funds to conduct further research and development and preclinical studies and clinical trials of our therapeutic candidates, to seek regulatory approvals for our therapeutic candidates and to manufacture and market products, if any, that are approved for commercial sale. As of September 30, 2017 and December 31, 2016, we had $22.9 million and $19.6 million in cash and cash equivalents, respectively. Based on our current operating plan, we believe that our available cash and cash equivalents as of September 30, 2017, when combined with the aggregate cash raised on October 27, 2017 and November 2, 2017 of approximately $11.2 million for the sale of common stock in the Offering, but excluding any potential proceeds from options exercises, is sufficient to fund our current operating plans into 2019. Our future capital requirements and the period for which we expect our existing resources to support our operations may vary significantly from what we expect. Our monthly spending levels vary based on new and ongoing development and corporate activities. Since the length of time and activities associated with successful development of our therapeutic candidates is highly uncertain, we are unable to estimate the actual funds we will require for development and any approved marketing and commercialization activities. To execute our business plan, we will need, among other things:

- to obtain the human and financial resources necessary to develop, test, obtain regulatory approval for, manufacture and market our therapeutic candidates;
- to build and maintain a strong intellectual property portfolio and avoid infringing the intellectual property of third parties;
- to establish and maintain successful licenses, collaborations and alliances;
- to satisfy the requirements of clinical trial protocols, including patient enrollment;
- to establish and demonstrate the clinical efficacy and safety of our therapeutic candidates;
- to obtain regulatory approvals;
- to manage our spending as costs and expenses increase due to preclinical studies and clinical trials, regulatory approvals, and commercialization;
- to obtain additional capital to support and expand our operations; and

- to market our products to achieve acceptance and use by the medical community in general.

If we are unable to obtain funding on a timely basis or on acceptable terms, we may have to delay, reduce or terminate our research and development programs and preclinical studies or clinical trials, if any, limit strategic opportunities or undergo reductions in our workforce or other corporate restructuring activities. We also could be required to seek funds through arrangements with collaborators or others that may require us to relinquish rights to some of our technology or therapeutic candidates that we would otherwise pursue on our own. We do not expect to realize revenue from product sales, milestone payments or royalties in the foreseeable future, if at all. Our revenue sources are, and will remain, extremely limited unless and until our therapeutic candidates are clinically tested, approved for commercialization and successfully marketed. To date, we have primarily financed our operations through the sale of equity securities, payments received in connection with our research collaboration, license, and option agreement with Purdue or as a primary contractor or as a subcontractor on government grants, and proceeds from our loan agreement with Hercules. We will be required to seek additional funding in the future and intend to do so through either collaborations, public or private equity offerings or debt financings, credit or loan facilities or a combination of one or more of these funding sources. Our ability to raise additional funds will depend on financial, economic and other factors, many of which are beyond our control. Additional funds may not be available to us on acceptable terms or at all. If we raise additional funds by issuing equity securities, our stockholders will suffer dilution and the terms of any financing may adversely affect the rights of our stockholders. In addition, as a condition to providing additional funds to us, future investors may demand, and may be granted, rights superior to those of existing stockholders. Debt financing, if available, may involve restrictive covenants limiting our flexibility in conducting future business activities, and, in the event of insolvency, debt holders would be repaid before holders of equity securities received any distribution of corporate assets.

Our quarterly operating results may fluctuate significantly or may fall below the expectations of investors or securities analysts, each of which may cause our stock price to fluctuate or decline.

We expect our operating results to be subject to quarterly fluctuations. Our net loss and other operating results will be affected by numerous factors, including:

- variations in the level of expense related to our therapeutic candidates or future development programs;
- results of clinical trials, or the addition or termination of clinical trials or funding support by us, or a future collaborator or licensing partner;
- our execution of any collaboration, licensing or similar arrangement, and the timing of payments we may make or receive under such existing or future arrangements or the termination or modification of any such existing or future arrangements;
- any intellectual property infringement lawsuit or opposition, interference or cancellation proceeding in which we may become involved;
- additions and departures of key personnel;
- strategic decisions by us or our competitors, such as acquisitions, divestitures, spin-offs, joint ventures, strategic investments or changes in business strategy;
- whether or not any of our therapeutic candidates receives regulatory approval, market acceptance and demand for such therapeutic candidates;
- regulatory developments affecting our therapeutic candidates or those of our competitors; and
- changes in general market and economic conditions.

If our quarterly operating results fall below the expectations of investors or securities analysts, the price of our common stock could decline substantially. Furthermore, any quarterly fluctuations in our operating results may, in turn, cause the price of our stock to fluctuate substantially.

We are dependent on Purdue for the successful development of therapeutic candidates in our collaboration arrangement with Purdue.

On December 2, 2016, Exicure entered into a research collaboration, option and license agreement with Purdue. As part of the agreement, Purdue has the option to obtain from us the full worldwide development and commercial rights to AST-005, an option to obtain three additional collaboration targets and a further option to obtain from us the full worldwide development and commercial rights to any therapeutic candidates developed targeting the three additional collaboration targets. Additionally, Purdue has rights of first offer to some potential collaboration targets. These rights of first offer are subject to limitations in time and scope. In connection with the Purdue Collaboration, we received a non-refundable development fee of $10 million. In addition, we are eligible to receive up to $776.5 million upon successful completion of certain research, regulatory and commercial sales milestones. There can be no assurance these milestones will be achieved as they are subject to highly significant risks and uncertainties, many of which are outside of our control. In the event a therapeutic candidate subject to the collaboration results in commercial sales, we are eligible to receive royalties ranging from the low single digits to a maximum of 10% on future net sales of such commercialized therapeutic candidates.

The success of our collaboration programs with Purdue depends largely upon the efforts of Purdue. Purdue has sole discretion in determining and directing the efforts and resources, including the ability to discontinue all efforts and resources, it applies to the development and, if approval is obtained, commercialization and marketing of the therapeutic candidates covered by the collaboration. Purdue may not be effective in obtaining approvals for the therapeutic candidates developed under the collaboration arrangement or marketing or arranging for necessary supply, manufacturing or distribution relationships for any approved products. Purdue may change its strategic focus or pursue alternative technologies in a manner that results in reduced, delayed or no revenue to us. Purdue has a variety of marketed products, and its own corporate objectives may not be consistent with our best interests. If Purdue fails to develop, obtain regulatory approval for or ultimately commercialize any therapeutic candidate under our collaboration or if Purdue terminates our collaboration, our business, financial condition, results of operations and prospects could be materially and adversely affected. In addition, any dispute or litigation proceedings we may have with Purdue in the future could delay development programs, create uncertainty as to ownership of intellectual property rights, distract management from other business activities and generate substantial expense.

If third parties on which we depend to conduct our preclinical studies and clinical trials do not perform as contractually required, fail to satisfy regulatory or legal requirements, or miss expected deadlines, our development program could be delayed with materially adverse effects on our business, financial condition, results of operations and prospects.

We rely on third party clinical investigators, contract research organizations ("CROs"), clinical data management organizations and consultants to design, conduct, supervise and monitor preclinical studies and clinical trials for our therapeutic candidates. Because we rely on third parties and do not have the ability to conduct preclinical studies or clinical trials independently, we have less control over the timing, quality and other aspects of preclinical studies and clinical trials than we would if we conducted them on our own. These investigators, CROs and consultants are not our employees and we have limited control over the amount of time and resources that they dedicate to our programs. These third parties may have contractual relationships with other entities, some of which may be our competitors, which may draw time and resources away from our programs. The third parties with which we contract might not be diligent, careful or timely in conducting our preclinical studies or clinical trials, resulting in the preclinical studies or clinical trials being delayed or unsuccessful.

If we cannot contract with acceptable third parties on commercially reasonable terms, or at all, or if these third parties do not carry out their contractual duties, satisfy legal and regulatory requirements for the conduct of preclinical studies or clinical trials or meet expected deadlines, our clinical development programs could be delayed and otherwise adversely affected. In all events, we are responsible for ensuring that each of our preclinical studies and clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. The FDA requires preclinical studies to be conducted in accordance with applicable Good Laboratory Practices ("GLPs") and clinical trials to be conducted in accordance with applicable FDA regulations and Good Clinical Practices ("GCPs), including requirements for conducting, recording and reporting the results of preclinical studies and clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and

confidentiality of clinical trial participants are protected. Our reliance on third parties that we do not control does not relieve us of these responsibilities and requirements. Any adverse development or delay in our preclinical studies or clinical trials could have a material adverse effect on our business, financial condition, results of operations and prospects.

Because we rely on third party manufacturing and supply partners, our supply of research and development, preclinical studies and clinical trial materials may become limited or interrupted or may not be of satisfactory quantity or quality.

We rely on third party partners to manufacture and supply the materials and components for our research and development, preclinical study and clinical trial supplies. We do not own manufacturing facilities or supply sources for such components and materials. Our manufacturing requirements include oligonucleotides and lipids. We procure our nonclinical toxicology and clinical development materials from a single source supplier on a purchase order basis. There can be no assurance that our supply of research and development, preclinical study and clinical trial therapeutic candidates and other materials will not be limited, interrupted, restricted in certain geographic regions or of satisfactory quality or continue to be available at acceptable prices. In particular, any replacement of our drug product manufacturers could require significant effort and expertise because there may be a limited number of qualified replacements.

The manufacturing process for a therapeutic candidate is subject to oversight by the FDA and foreign regulatory authorities. Suppliers and manufacturers must meet applicable manufacturing requirements and undergo rigorous facility and process validation tests required by regulatory authorities in order to comply with regulatory requirements, such as current Good Manufacturing Practices ("cGMPs"). In the event that any of our suppliers or manufacturers fails to comply with such requirements or to perform its obligations to us in relation to quality, timing or otherwise, or if our supply of components or other materials becomes limited or interrupted for other reasons, we may be forced to manufacture the materials ourselves, for which we currently do not have the capabilities or resources, or enter into an agreement with another third party, which we may not be able to do on reasonable terms, if at all. In some cases, the technical skills or technology required to manufacture our therapeutic candidates may be unique or proprietary to the original manufacturer and we may have difficulty, or there may be contractual restrictions prohibiting us from, transferring such skills or technology to another third party and a feasible alternative may not exist. These factors would increase our reliance on such manufacturer or require us to obtain a license from such manufacturer in order to have another third party manufacture our therapeutic candidates. If we are required to change manufacturers for any reason, we will be required to verify that the new manufacturer maintains facilities and procedures that comply with quality standards and with all applicable regulations. The delays associated with the verification of a new manufacturer could negatively affect our ability to develop therapeutic candidates in a timely manner or within budget.

We expect to continue to rely on third party manufacturers if we receive regulatory approval for any therapeutic candidate. To the extent that we have existing, or enter into future, manufacturing arrangements with third parties, we will depend on these third parties to perform their obligations in a timely manner consistent with contractual and regulatory requirements, including those related to quality control and assurance. If we are unable to obtain or maintain third party manufacturing for therapeutic candidates, or to do so on commercially reasonable terms, we may not be able to develop and commercialize our therapeutic candidates successfully. Our or a third party's failure to execute on our manufacturing requirements and comply with cGMP could adversely affect our business in a number of ways, including:

- an inability to initiate or continue preclinical studies or clinical trials of our therapeutic candidates under development;
- delay in submitting regulatory applications, or receiving regulatory approvals, for therapeutic candidates;
- loss of the cooperation of a future collaborator;
- subjecting manufacturing facilities of our therapeutic candidates to additional inspections by regulatory authorities;

- requirements to cease distribution or to recall batches of our therapeutic candidates; and
- in the event of approval to market and commercialize a therapeutic candidate, an inability to meet commercial demands for our therapeutics.

We may not successfully engage in strategic transactions, including any collaborations we seek, which could adversely affect our ability to develop and commercialize therapeutic candidates, impact our cash position, increase our expense, and present significant distractions to our management.

From time to time, we may consider strategic transactions, such as collaborations, acquisitions of companies, asset purchases and out- or in-licensing of therapeutic candidates or technologies. In particular, we will evaluate and, if strategically attractive, seek to enter into collaborations, including with major biotechnology or pharmaceutical companies. The competition for collaborators is intense, and the negotiation process is time-consuming and complex. Any new collaboration may be on terms that are not optimal for us, and we may be unable to maintain any new collaboration if, for example, development or approval of a therapeutic candidate is delayed, sales of an approved therapeutic candidate do not meet expectations or the collaborator terminates the collaboration. Any such collaboration, or other strategic transaction, may require us to incur non-recurring or other charges, increase our near- and long-term expenditures and pose significant integration or implementation challenges or disrupt our management or business. These transactions would entail numerous operational and financial risks, including exposure to unknown liabilities, disruption of our business and diversion of our management's time and attention in order to manage a collaboration or develop acquired therapeutics, therapeutic candidates or technologies, incurrence of substantial debt or dilutive issuances of equity securities to pay transaction consideration or costs, higher than expected collaboration, acquisition or integration costs, write-downs of assets or goodwill or impairment charges, increased amortization expenses, difficulty and cost in facilitating the collaboration or combining the operations and personnel of any acquired business, impairment of relationships with key suppliers, manufacturers or customers of any acquired business due to changes in management and ownership and the inability to retain key employees of any acquired business. Accordingly, although there can be no assurance that we will undertake or successfully complete any transactions of the nature described above, any transactions that we do complete may be subject to the foregoing or other risks and may have a material adverse effect on our business, results of operations, financial condition and prospects. Conversely, any failure to enter into any collaboration or other strategic transaction that would be beneficial to us could delay the development and potential commercialization of our therapeutic candidates and have a negative impact on the competitiveness of any therapeutic candidate that reaches market.

We face competition from entities that have developed or may develop therapeutic candidates for our target disease indications, including companies developing novel treatments and technology platforms based on modalities and technology similar to ours. If these companies develop technologies, including delivery technologies, or therapeutic candidates more rapidly than we do or their technologies are more effective, our ability to develop and successfully commercialize therapeutic candidates may be adversely affected.

The development and commercialization of therapeutic candidates is highly competitive. We compete with a number of multinational pharmaceutical companies and specialized biotechnology companies, as well as technology being developed at universities and other research institutions. Our competitors have developed, are developing or will develop therapeutic candidates and processes competitive with our therapeutic candidates. Competitive therapeutic treatments include those that have already been approved and accepted by the medical community and any new treatments that enter the market. We believe that a significant number of therapeutics are currently under development, and may become commercially available in the future, for the treatment of conditions for which we may try to develop therapeutic candidates. There is intense and rapidly evolving competition in the biotechnology, pharmaceutical and oligonucleotide therapeutics fields. While we believe that our SNA technology, its associated intellectual property and our scientific and technical know-how give us a competitive advantage in this space, competition from many sources remains. Our competitors include larger and better funded pharmaceutical, biotechnology and oligonucleotide therapeutics companies. Moreover, we also compete with current and future therapeutics developed at universities and other research institutions.

We are aware of several companies that are developing oligonucleotide delivery platforms and oligonucleotide-based therapeutics. These competitors include Ionis Pharmaceuticals, Inc., Alnylam Pharmaceuticals, Inc., Dicerna

Pharmaceuticals, Inc., Arbutus Biopharma Corp., Wave Life Sciences Ltd., Dynavax Technologies Corp., Idera Pharmaceuticals, Inc., Mologen AG, and Checkmate Pharmaceuticals, Inc. These and other competitors compete with us in recruiting scientific and managerial talent, and for funding from pharmaceutical companies.

Our success will partially depend on our ability to develop and protect therapeutics that are safer and more effective than competing therapeutics. Our commercial opportunity and success will be reduced or eliminated if competing therapeutics are safer, more effective, or less expensive than the therapeutics we develop.

If our lead therapeutic candidates are approved for the indications we are currently pursuing, they will compete with a range of therapeutic treatments that are either in development or currently marketed. A number of therapeutics for treating psoriasis and cancers are on the market or in clinical development. For the treatment of psoriasis, marketed therapies range from small molecules like topical steroids to biologics, such as AbbVie Inc.'s adalimumab. In addition, numerous compounds are in clinical development for psoriasis treatment. With respect to immunogenic cancers such as melanoma, the most common treatments are chemotherapeutic compounds, radiation therapy and now immunotherapeutic antibodies such as ipilimumab, atezolizumab and pembrolizumab.

Many of our competitors have significantly greater financial, technical, manufacturing, marketing, sales and supply resources or experience than we do. If we successfully obtain approval for any therapeutic candidate, we will face competition based on many different factors, including the safety and effectiveness of our therapeutics, the ease with which our therapeutics can be administered and the extent to which patients accept relatively new routes of administration, the timing and scope of regulatory approvals for these therapeutics, the availability and cost of manufacturing, marketing and sales capabilities, price, reimbursement coverage and patent position. Competing therapeutics could present superior treatment alternatives, including by being more effective, safer, less expensive or marketed and sold more effectively than any therapeutics we may develop. Competitive therapeutics may make any therapeutics we develop obsolete or noncompetitive before we recover the expense of developing and commercializing our therapeutic candidates. Such competitors could also recruit our employees, which could negatively impact our level of expertise and our ability to execute our business plan.

The market may not be receptive to our therapeutic candidates based on a novel therapeutic modality, and we may not generate any future revenue from the sale or licensing of therapeutic candidates.

Even if approval is obtained for a therapeutic candidate, we may not generate or sustain revenue from sales of the product due to factors such as whether the product can be sold at a competitive cost and otherwise accepted in the market. The therapeutic candidates that we are developing are based on our SNA technology. Market participants with significant influence over acceptance of new treatments, such as physicians and third party payors, may not adopt a treatment based on SNA technology, and we may not be able to convince the medical community and third party payors to accept and use, or to provide favorable reimbursement for, any therapeutic candidates developed by us or any current or future collaborators. Market acceptance of our therapeutic candidates will depend on, among other factors:

- the timing of our receipt of any marketing and commercialization approvals;
- the terms of any approvals and the countries in which approvals are obtained;
- the safety and efficacy of our therapeutic candidates;
- the prevalence and severity of any adverse side effects associated with our therapeutic candidates;
- limitations or warnings contained in any labeling approved by the FDA or other regulatory authority;
- relative convenience and ease of administration of our therapeutic candidates;
- the willingness of patients to accept any new methods of administration;
- the success of our physician education programs;
- the availability of adequate government and third party payor reimbursement;

- the pricing of our products, particularly as compared to alternative treatments; and
- availability of alternative effective treatments for indications our therapeutic candidates are intended to treat and the relative risks, benefits and costs of those treatments.

With our focus on SNAs, these risks may increase to the extent the space becomes more competitive or less favored in the commercial marketplace. Additional risks apply in relation to any disease indications we may pursue which are classified as rare diseases and allow for orphan drug designation by regulatory agencies in major commercial markets, such as the U.S., Europe and Japan. Because of the small patient population for a rare disease, if pricing is not approved or accepted in the market at an appropriate level for an approved product with orphan drug designation, such therapeutic may not generate enough revenue to offset costs of development, manufacturing, marketing and commercialization despite any benefits received from the orphan drug designation, such as market exclusivity, assistance in clinical trial design or a reduction in user fees or tax credits related to development expense. Market size is also a variable in disease indications not classified as rare. Our estimates regarding potential market size for any indication may be materially different from what we discover to exist at the time we commence commercialization, if any, for a therapeutic, which could result in significant changes in our business plan and have a material adverse effect on our business, financial condition, results of operations and prospects.

If a therapeutic candidate that has orphan drug designation subsequently receives the first FDA approval for the indication for which it has such designation, the therapeutic candidate is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same therapeutic candidate for the same indication, except in very limited circumstances, for seven years. Orphan drug exclusivity, however, could also block the approval of one of our therapeutic candidates for seven years if a competitor obtains approval of the same therapeutic candidate as defined by the FDA or if our therapeutic candidate is determined to be contained within the competitor's therapeutic candidate for the same indication or disease.

As in the U.S., we may apply for designation of a therapeutic candidate as an orphan drug for the treatment of a specific indication in the European Union before the application for marketing authorization is made. Sponsors of orphan drugs in the European Union can enjoy economic and marketing benefits, including up to ten years of market exclusivity for the approved indication unless certain exceptions apply.

Any inability to attract and retain qualified key management and technical personnel would impair our ability to implement our business plan.

Our success largely depends on the continued service of key management and other specialized personnel, including David A. Giljohann, Ph.D., our Chief Executive Officer, David S. Snyder, our Chief Financial Officer, and Ekambar Kandimalla, Ph.D., our Chief Scientific Officer. The loss of one or more members of our management team or other key employees or advisors could delay our research and development programs and materially harm our business, financial condition, results of operations and prospects. The relationships that our key managers have cultivated within our industry make us particularly dependent upon their continued employment with us. We are dependent on the continued service of our technical personnel because of the highly technical nature of our therapeutic candidates and our technology and the specialized nature of the regulatory approval process. Because our management team and key employees are not obligated to provide us with continued service, they could terminate their employment with us at any time without penalty. We do not maintain key person life insurance policies on any of our management team members or key employees. Our future success will depend in large part on our continued ability to attract and retain highly qualified scientific, technical and management personnel, as well as personnel with expertise in clinical testing, manufacturing, governmental regulation and commercialization. We face competition for personnel from other companies, universities, public and private research institutions, government entities and other organizations.

If our therapeutic candidates advance into clinical trials, we may experience difficulties in managing our growth and expanding our operations.

We have limited experience in therapeutic development and limited experience with clinical trials of therapeutic candidates. As our therapeutic candidates enter and advance through preclinical studies and clinical trials, we will need to expand our development, regulatory and manufacturing capabilities or contract with other organizations to

provide these capabilities for us. In the future, we expect to have to manage additional relationships with collaborators or partners, suppliers and other organizations. Our ability to manage our operations and future growth will require us to continue to improve our operational, financial and management controls, reporting systems and procedures. We may not be able to implement improvements to our management information and control systems in an efficient or timely manner and may discover deficiencies in existing systems and controls.

If any of our therapeutic candidates are approved for marketing and commercialization and we are unable to develop sales, marketing and distribution capabilities on our own or enter into agreements with third parties to perform these functions on acceptable terms, we will be unable to successfully commercialize any such future therapeutics.

We currently have no sales, marketing or distribution capabilities or experience. If any of our therapeutic candidates is approved, we will need to develop internal sales, marketing and distribution capabilities to commercialize such therapeutics, which would be expensive and time-consuming, or enter into collaborations with third parties to perform these services. If we decide to market our approved therapeutics directly, we will need to commit significant financial and managerial resources to develop a marketing and sales force with technical expertise and supporting distribution, administration and compliance capabilities. If we rely on third parties with such capabilities to market our approved therapeutics or decide to co-promotetherapeutics with collaborators, we will need to establish and maintain marketing and distribution arrangements with third parties, and there can be no assurance that we will be able to enter into such arrangements on acceptable terms or at all. In entering into third party marketing or distribution arrangements, any revenue we receive will depend upon the efforts of the third parties and there can be no assurance that such third parties will establish adequate sales and distribution capabilities or be successful in gaining market acceptance of any approved therapeutic. If we are not successful in commercializing any therapeutic approved in the future, either on our own or through third parties, our business, financial condition, results of operations and prospects could be materially and adversely affected.

If we fail to comply with U.S. or foreign regulatory requirements, regulatory authorities could withhold marketing or commercialization approvals, limit or withdraw any marketing or commercialization approvals we may receive and subject us to other penalties that could materially harm our business.

We and our therapeutic candidates, as well as our suppliers, contract manufacturers, distributors, and contract testing laboratories are subject to extensive regulation by governmental authorities in the European Union, the U.S., and other countries, with the regulations differing from country to country.

If we or current or future collaborators, manufacturers or service providers fail to comply with applicable requirements, these regulatory authorities could refuse to issue necessary approvals for marketing and commercialization. Even if we receive marketing and commercialization approval of a therapeutic candidate, we and our third party service providers will be subject to continuing regulatory requirements, including a broad array of regulations related to establishment, registration and product listing, manufacturing processes, risk management measures, quality and pharmacovigilance systems, pre- and post-approval clinical data, labeling, advertising and promotional activities for such therapeutic, record keeping, distribution, and import and export of therapeutics for any therapeutic for which we obtain marketing approval. We are required to submit safety and other post market information and reports and are subject to continuing regulatory review, including in relation to adverse patient experiences with the therapeutic and clinical results that are reported after a therapeutic is made commercially available, both in the U.S. and any foreign jurisdiction in which we seek regulatory approval. The FDA and certain foreign regulatory authorities, such as the EMA, have significant post-market authority, including the authority to require labeling changes based on new safety information and to require post-market studies or clinical trials to evaluate safety risks related to the use of a therapeutic or to require withdrawal of the therapeutic from the market. The FDA also has the authority to require a Risk Evaluation and Mitigation Strategies ("REMS") plan either before or after approval, which may impose further requirements or restrictions on the distribution or use of an approved therapeutic. The EMA now routinely requires risk management plans ("RMPs"), as part of the marketing authorization application process, and such plans must be continually modified and updated throughout the lifetime of the product as new information becomes available. In addition, the relevant governmental authority of any EU member state can request an RMP whenever there is a concern about the risk/benefit balance of the product.

The manufacturer and manufacturing facilities we use to make a future therapeutic, if any, will also be subject to periodic review and inspection by the FDA and other regulatory agencies, including for continued compliance with cGMP requirements. The discovery of any new or previously unknown problems with our third party manufacturers, manufacturing processes or facilities may result in restrictions on the therapeutic, manufacturer or facility, including withdrawal of the therapeutic from the market. If we rely on third party manufacturers, we will not have control over compliance with applicable rules and regulations by such manufacturers. Any product promotion and advertising will also be subject to regulatory requirements and continuing regulatory review. If we or our future collaborators, manufacturers or service providers fail to comply with applicable continuing regulatory requirements in the U.S. or foreign jurisdictions in which we seek to market our therapeutics, we or they may be subject to, among other things, fines, warning and untitled letters, clinical holds, delay or refusal by the FDA or foreign regulatory authorities to approve pending applications or supplements to approved applications, suspension, refusal to renew or withdrawal of regulatory approval, recalls, seizures or administrative detention of products, refusal to permit the import or export of therapeutics, operating restrictions, inability to participate in government programs including Medicare and Medicaid, and total or partial suspension of production or distribution, injunction, restitution, disgorgement, debarment, civil and criminal penalties and criminal prosecution.

Price controls imposed in foreign markets may adversely affect our future profitability.

In some countries, particularly member states of the European Union, the pricing of prescription drugs is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after receipt of marketing approval for a therapeutic. In addition, there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. Political, economic and regulatory developments may further complicate pricing and reimbursement negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various European Union member states and parallel distribution, or arbitrage between low-priced and high-priced member states, can further reduce prices. In some countries, we or current or future collaborators may be required to conduct a clinical trial or other studies that compare the cost-effectiveness of our SNA therapeutic candidates to other available therapies in order to obtain or maintain reimbursement or pricing approval. Publication of discounts by third party payors or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries. If reimbursement of any therapeutic candidate approved for marketing is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business, financial condition, results of operations or prospects could be adversely affected.

Our business entails a significant risk of product liability and our inability to obtain sufficient insurance coverage could have a material adverse effect on our business, financial condition, results of operations or prospects.

Our business exposes us to significant product liability risks inherent in the development, testing, manufacturing and marketing of therapeutic treatments. Product liability claims could delay or prevent completion of our development programs. If we succeed in marketing therapeutics, such claims could result in an investigation by certain regulatory authorities, such as the FDA or foreign regulatory authorities, of the safety and effectiveness of our therapeutics, our manufacturing processes and facilities or our marketing programs and potentially a recall of our therapeutics or more serious enforcement action, limitations on the approved indications for which they may be used or suspension or withdrawal of approvals. Regardless of the merits or eventual outcome, liability claims may also result in decreased demand for our therapeutics, injury to our reputation, costs to defend the related litigation, a diversion of management's time and our resources, substantial monetary awards to trial participants or patients and a decline in our stock price. We currently have product liability insurance that we believe is appropriate for our stage of development and may need to obtain higher levels of product liability insurance prior to marketing any of our therapeutic candidates. Any insurance we have or may obtain may not provide sufficient coverage against potential liabilities. Furthermore, clinical trial and product liability insurance is becoming increasingly expensive. As a result, we may be unable to obtain sufficient insurance at a reasonable cost to protect us against losses caused by product liability claims that could have a material adverse effect on our business.

Our employees may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements which could have an adverse effect on our business.

We are exposed to the risk of employee fraud or other misconduct. Misconduct by employees could include, but is not limited to, intentional failures to comply with FDA regulations, applicable laws, regulations, guidance or codes of conduct set by foreign governmental authorities or self-regulatory industry organizations, or provide accurate information to any governmental authorities, such as the FDA, comply with manufacturing standards we may establish, comply with federal and state healthcare fraud and abuse laws and regulations, report financial information or data accurately or disclose unauthorized activities to us.

In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws, regulations, guidance and codes of conduct intended to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws, regulations, guidance and codes of conduct may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements.

Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions, including debarment or disqualification of those employees from participation in FDA-regulated activities, and serious harm to our reputation. This could include violations of the U.S. federal Health Insurance Portability and Accountability Act of 1997 ("HIPAA"), as amended by the Health Information Technology for Economic and Clinical Health Act ("HITECH"), other U.S. federal and state law, and requirements of non-U.S. jurisdictions, including the European Union Data Protection Directive.

It is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws, regulations, guidance or codes of conduct. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

Compliance with governmental regulations regarding the treatment of animals used in research could increase our operating costs, which would adversely affect the commercialization of our technology.

The Animal Welfare Act ("AWA"), is the federal law that covers the treatment of certain animals used in research. Currently, the AWA imposes a wide variety of specific regulations that govern the humane handling, care, treatment and transportation of certain animals by producers and users of research animals, most notably relating to personnel, facilities, sanitation, cage size, and feeding, watering and shipping conditions. Third parties with whom we contract are subject to registration, inspections and reporting requirements under the AWA. Furthermore, some states have their own regulations, including general anti-cruelty legislation, which establish certain standards in handling animals. Comparable rules, regulations, and or obligations exist in many foreign jurisdictions. If we or our contractors fail to comply with regulations concerning the treatment of animals used in research, we may be subject to fines and penalties and adverse publicity, and our operations could be adversely affected.

Our internal computer systems, or those of our CROs or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our therapeutic development programs.

Despite the implementation of security measures, our internal computer systems and those of our CROs and other contractors and consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. Such events could cause interruptions of our operations. For instance, the loss of preclinical study or clinical trial data involving our therapeutic candidates could result in delays in our development and regulatory filing efforts and significantly increase our costs. In addition, theft or other exposure of data may interfere with our ability to protect our intellectual property, trade secrets, and other information critical to our operations. We can provide no assurances that certain sensitive and proprietary information relating to one or more of our therapeutic candidates has not been, or will not in the future be, compromised. Although we have invested resources to enhance the security of our computer systems, there can be no assurances we will not experience additional unauthorized intrusions into our computer systems, or those of our

CROs and other contractors and consultants, that we will successfully detect future unauthorized intrusions in a timely manner, or that future unauthorized intrusions will not result in material adverse effects on our financial condition, reputation, or business prospects. Payments related to the elimination of ransomware may materially affect our financial condition and results of operations.

Certain data breaches must also be reported to affected individuals and the government, and in some cases to the media, under provisions of HIPAA, as amended by HITECH, other U.S. federal and state law, and requirements of non-U.S. jurisdictions, including the European Union Data Protection Directive, and financial penalties may also apply.

To the extent that any disruption or security breach were to result in a loss of, or damage to, our data, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the development of our therapeutic candidates could be delayed.

If we do not comply with laws regulating the protection of the environment and health and human safety, our business could be adversely affected.

Our research, development and manufacturing involve the use of hazardous materials and various chemicals. We maintain quantities of various flammable and toxic chemicals in our facilities in Skokie, Illinois that are required for our research, development and manufacturing activities. We are subject to federal, state and local laws and regulations governing the use, manufacture, storage, handling and disposal of these hazardous materials. We believe our procedures for storing, handling and disposing these materials in our Skokie facilities comply with the relevant guidelines of Skokie, the state of Illinois, and the Occupational Safety and Health Administration of the U.S. Department of Labor. Although we believe that our safety procedures for handling and disposing of these materials comply with the standards mandated by applicable regulations, the risk of accidental contamination or injury from these materials cannot be eliminated. If an accident occurs, we could be held liable for resulting damages, which could be substantial. We are also subject to numerous environmental, health and workplace safety laws and regulations, including those governing laboratory procedures, exposure to blood-borne pathogens and the handling of animals and biohazardous materials. Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of these materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological or hazardous materials. Additional federal, state and local laws and regulations affecting our operations may be adopted in the future. We may incur substantial costs to comply with, and substantial fines or penalties if we violate, any of these laws or regulations.

Our information technology systems could face serious disruptions that could adversely affect our business.

Our information technology and other internal infrastructure systems, including corporate firewalls, servers, leased lines and connection to the Internet, face the risk of systemic failure that could disrupt our operations. A significant disruption in the availability of our information technology and other internal infrastructure systems could cause interruptions and delays in our research and development work.

Our current operations are concentrated in one location and any events affecting this location may have material adverse consequences.

Our current operations are located in our facilities situated in Skokie, Illinois. Any unplanned event, such as flood, fire, explosion, earthquake, extreme weather condition, medical epidemics, power shortage, telecommunication failure or other natural or man-made accidents or incidents that result in us being unable to fully utilize the facilities, may have a material adverse effect on our ability to operate our business, particularly on a daily basis, and have significant negative consequences on our financial and operating conditions. Loss of access to these facilities may result in increased costs, delays in the development of our therapeutic candidates or interruption of our business operations. As part of our risk management policy, we maintain insurance coverage at levels that we believe are appropriate for our business. However, in the event of an accident or incident at these facilities, we cannot assure you that the amounts of insurance will be sufficient to satisfy any damages and losses. If our facilities are unable to operate because of an accident or incident or for any other reason, even for a short period of time, any

or all of our research and development programs may be harmed. Any business interruption may have a material adverse effect on our business, financial position, results of operations and prospects.

The investment of our cash, cash equivalents and fixed income marketable securities is subject to risks which may cause losses and affect the liquidity of these investments.

As of September 30, 2017 and December 31, 2016, we had $22.9 million and $19.6 million in cash and cash equivalents, respectively. We historically have invested excess cash in certificates of deposit or money market mutual funds which invest exclusively in U.S. government or U.S. government agency securities. These investments are subject to general credit, liquidity, market and interest rate risks, including potential future impacts similar to the impact of U.S. sub-prime mortgage defaults that have affected various sectors of the financial markets and caused credit and liquidity issues. We may realize losses in the fair value of these investments, an inability to access cash in these investments for a potentially meaningful period, or a complete loss of these investments, which would have a negative effect on our financial statements.

In addition, should our investments cease paying or reduce the amount of interest paid to us, our interest income would suffer. The market risks associated with our investment portfolio may have an adverse effect on our results of operations, liquidity and financial condition.

Changes in accounting rules and regulations, or interpretations thereof, could result in unfavorable accounting charges or require us to change our compensation policies.

Accounting methods and policies for biotechnology companies, including policies governing revenue recognition, research and development and related expenses, and accounting for stock-based compensation, are subject to review, interpretation and guidance from our auditors and relevant accounting authorities, including the SEC. Changes to accounting methods or policies, or interpretations thereof, may require us to reclassify, restate or otherwise change or revise our historical financial statements, including those contained in this Quarterly Report on Form 10-Q.

Our business may be affected by litigation and government investigations.

We may from time to time receive inquiries and subpoenas and other types of information requests from government authorities and others and we may become subject to claims and other actions related to our business activities. While the ultimate outcome of investigations, inquiries, information requests and legal proceedings is difficult to predict, defense of litigation claims can be expensive, time-consuming and distracting, and adverse resolutions or settlements of those matters may result in, among other things, modification of our business practices, costs and significant payments, any of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Risks Related to Intellectual Property

If we are not able to obtain and enforce patent protection for our technology or therapeutic candidates, development and commercialization of our therapeutic candidates may be adversely affected.

Our success depends in part on our ability to obtain and maintain patents and other forms of intellectual property rights, including in-licenses of intellectual property rights of others, for our therapeutic candidates, methods used to manufacture our therapeutic candidates and methods for treating patients using our therapeutic candidates, as well as our ability to preserve our trade secrets, to prevent third parties from infringing upon our proprietary rights and to operate without infringing upon the proprietary rights of others. As of September 30, 2017, our patent portfolio consists of over 45 issued patents and allowed patent applications and over 125 pending patent applications. We may not be able to apply for patents on certain aspects of our therapeutic candidates in a timely fashion or at all. Our existing issued and granted patents and any future patents we obtain may not be sufficiently broad to prevent others from using our technology or from developing competing therapeutics and technology. There is no guarantee that any of our pending patent applications will result in issued or granted patents, that any of our issued or granted patents will not later be found to be invalid or unenforceable or that any issued or granted patents will include claims that are sufficiently broad to cover our therapeutic candidates or to provide meaningful

protection from our competitors. Moreover, the patent position of pharmaceutical and biotechnology companies can be highly uncertain because it involves complex legal and factual questions. We will be able to protect our proprietary rights from unauthorized use by third parties only to the extent that our current and future proprietary technology and therapeutic candidates are covered by valid and enforceable patents or are effectively maintained as trade secrets. If third parties disclose or misappropriate our proprietary rights, it may materially and adversely impact our position in the market.

The U.S. Patent and Trademark Office ("USPTO"), and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process. There are situations in which noncompliance can result in abandonment or lapse of a patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, competitors might be able to enter the market earlier than would otherwise have been the case. The standards applied by the USPTO and foreign patent offices in granting patents are not always applied uniformly or predictably. For example, there is no uniform worldwide policy regarding patentable subject matter or the scope of claims allowable in biotechnology and pharmaceutical patents. As such, we do not know the degree of future protection that we will have on our proprietary therapeutics and technology. While we will endeavor to try to protect our therapeutic candidates with intellectual property rights such as patents, as appropriate, the process of obtaining patents is time-consuming, expensive and sometimes unpredictable.

In addition, there are numerous recent changes to the patent laws and proposed changes to the rules of the USPTO, which may have a significant impact on our ability to protect our technology and enforce our intellectual property rights. For example, the Leahy-Smith America Invents Act enacted in 2011, involves significant changes in patent legislation. The U.S. Supreme Court has ruled on several patent cases in recent years, some of which cases either narrow the scope of patent protection available in certain circumstances or weaken the rights of patent owners in certain situations. The 2013 decision by the Supreme Court in *Association for Molecular Pathology v. Myriad Genetics, Inc.* precludes a claim to a nucleic acid having a stated nucleotide sequence that is identical to a sequence found in nature and unmodified. We currently are not aware of an immediate impact of this decision on our patents or patent applications because we are developing oligonucleotide therapeutics which contain modifications that we believe are not found in nature. However, this decision has yet to be clearly interpreted by courts and by the USPTO. We cannot assure you that the interpretations of this decision or subsequent rulings will not adversely impact our patents or patent applications. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways that may weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

Once granted, patents may remain open to opposition, interference, re-examination, post-grant review, inter partes review, nullification or derivation action in court or before patent offices or similar proceedings for a given period after allowance or grant, during which time third parties can raise objections against such initial grant. In the course of such proceedings, which may continue for a protracted period of time, the patent owner may be compelled to limit the scope of the allowed or granted claims thus attacked, or may lose the allowed or granted claims altogether. In addition, there can be no assurance that:

- Others will not or may not be able to make, use or sell compounds that are the same as or similar to our therapeutic candidates but that are not covered by the claims of the patents that we own or license.
- We or our licensors, or any current or future collaborators, are the first to make the inventions covered by each of our issued patents and pending patent applications that we own or license.
- We or our licensors, or any current or future collaborators, are the first to file patent applications covering certain aspects of our inventions.
- Others will not independently develop similar or alternative technologies or duplicate any of our technology without infringing our intellectual property rights.

- A third party will not challenge our patents and, if challenged, a court may not hold that our patents are valid, enforceable and infringed.
- Any issued patents that we own or have licensed will provide us with any competitive advantages, or will not be challenged by third parties.
- We will develop additional proprietary technologies that are patentable.
- The patents of others will not have an adverse effect on our business.
- Our competitors will not conduct research and development activities in countries where we lack enforceable patent rights and then use the information learned from such activities to develop competitive therapeutics for sale in our major commercial markets.

We currently license patent rights from Northwestern University and may in the future license patent rights from third party owners or licensees. If Northwestern University or such other owners or licensees do not properly or successfully obtain, maintain or enforce the patents underlying such licenses, or if they retain or license to others any competing rights, our competitive position and business prospects may be adversely affected.

We do, and will continue to, rely on intellectual property rights licensed from third parties to protect our technology. We are a party to a number of licenses that give us rights to third party intellectual property that is necessary or useful for our business. In particular, we have a license from NU, which provides us the exclusive worldwide right under certain patents and patent applications owned by NU to exploit therapeutics and processes using nanoparticles, nanotechnology, microtechnology and nanomaterial-based constructs as therapeutics or accompanying therapeutics as a means of administration. We may also license additional third party intellectual property in the future. Our success will depend in part on the ability of our licensors to obtain, maintain and enforce patent protection for our licensed intellectual property, and in particular, for those patents to which we have secured exclusive rights. Our licensors may not successfully prosecute the patent applications licensed to us. Even if patents issue or are granted, our licensors may fail to maintain these patents, may determine not to pursue litigation against other companies that are infringing these patents, or may pursue litigation less aggressively than we would. Further, we may not obtain exclusive rights, which would allow for third parties to develop competing therapeutics. Without protection for, or exclusive rights to, the intellectual property we license, other companies might be able to offer substantially identical therapeutics for sale, which could adversely affect our competitive business position and harm our business prospects. In addition, the U.S. government has certain rights to the inventions covered by the patent rights licensed to us by third parties and NU, as an academic research and medical center, has reserved the right to practice the patent rights it has licensed to us (i) for research, teaching and/or other educationally related purposes (including the right to distribute materials for such purposes) and (ii) for use in the field of diagnostics (including theradiagnostics) and in any field other than the field of use licensed to us.

Other companies or organizations may challenge our or our licensors' patent rights or may assert patent rights that prevent us from developing and commercializing our therapeutics.

Oligonucleotide and SNA-based therapeutics are a relatively new scientific field. We have obtained grants and issuances of SNA therapeutic patents and have licensed many of these patents from a third party on an exclusive basis for therapeutics applications. The issued patents and pending patent applications in the U.S. and in key markets around the world that we own or license claim many different methods, compositions and processes relating to the discovery, development, manufacture and commercialization of SNA therapeutics. Specifically, we own and have licensed a portfolio of patents, patent applications and other intellectual property covering SNA compositions of matter as well as their methods of use.

As the field of SNA therapeutics matures, patent applications are being processed by national patent offices around the world. There is uncertainty about which patents will issue, and, if they do, as to when, to whom, and with what claims. In addition, third parties may attempt to invalidate our intellectual property rights. Even if our rights are not directly challenged, disputes could lead to the weakening of our intellectual property rights. Our defense against any attempt by third parties to circumvent or invalidate our intellectual property rights could be costly to us,

could require significant time and attention of our management and could have a material adverse effect on our business and our ability to successfully compete.

There are many issued and pending patents that claim aspects of oligonucleotide chemistry and modifications that we may need to apply to our SNA therapeutic candidates. There are also many issued patents that claim targeting genes or portions of genes that may be relevant for SNA therapeutics we wish to develop. Thus, it is possible that one or more organizations will hold patent rights to which we will need a license. If those organizations refuse to grant us a license to such patent rights on reasonable terms, we may not be able to market therapeutics or perform research and development or other activities covered by these patents.

We may be unable to protect our intellectual property rights throughout the world.

Obtaining a valid and enforceable issued or granted patent covering our technology in the U.S. and worldwide can be extremely costly. In jurisdictions where we have not obtained patent protection, competitors may use our technology to develop their own therapeutics and, further, may export otherwise infringing therapeutics to territories where we have patent protection, but where it is more difficult to enforce a patent as compared to the U.S. Competitor therapeutics may compete with our future therapeutics in jurisdictions where we do not have issued or granted patents or where our issued or granted patent claims or other intellectual property rights are not sufficient to prevent competitor activities in these jurisdictions. The legal systems of certain countries, particularly certain developing countries, make it difficult to enforce patents and such countries may not recognize other types of intellectual property protection, particularly that relating to biotechnology and pharmaceuticals. This could make it difficult for us to prevent the infringement of our patents or marketing of competing therapeutics in violation of our proprietary rights generally in certain jurisdictions. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial cost and divert our efforts and attention from other aspects of our business.

We generally file a provisional patent application first, also known as a priority filing, at the USPTO. An international application under the Patent Cooperation Treaty ("PCT") is usually filed within twelve months after the priority filing. Based on the PCT filing, national and regional patent applications may be filed in the U.S., European Union, Japan, Australia and Canada and, depending on the individual case, also in any or all of, *inter alia*, China, India, South Korea, and Mexico. We have so far not filed for patent protection in all national and regional jurisdictions where such protection may be available. In addition, we may decide to abandon national and regional patent applications before grant. Finally, the grant proceeding of each national or regional patent is an independent proceeding which may lead to situations in which applications might in some jurisdictions be refused by the relevant registration authorities, while granted by others. It is also quite common that depending on the country, various scopes of patent protection may be granted on the same therapeutic candidate or technology.

The laws of some jurisdictions do not protect intellectual property rights to the same extent as the laws in the U.S., and many companies have encountered significant difficulties in protecting and defending such rights in such jurisdictions. If we or our licensors encounter difficulties in protecting, or are otherwise precluded from effectively protecting, the intellectual property rights important for our business in such jurisdictions, the value of these rights may be diminished and we may face additional competition from others in those jurisdictions. Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we or any of our licensors are forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position in the relevant jurisdiction may be impaired and our business and results of operations may be adversely affected.

We or our licensors, or any current or future strategic partners, may become subject to third party claims or litigation alleging infringement of patents or other proprietary rights or seeking to invalidate patents or other proprietary rights, and we may need to resort to litigation to protect or enforce our patents or other proprietary rights, all of which could be costly, time consuming, delay or prevent the development and commercialization of our therapeutic candidates, or put our patents and other proprietary rights at risk.

We or our licensors, or any current or future strategic partners, may be subject to third party claims for infringement or misappropriation of patent or other proprietary rights. We are generally obligated under our license

agreements to indemnify and hold harmless our licensors for damages arising from intellectual property infringement by us. If we or our licensors, or any current or future strategic partners, are found to infringe a third party patent or other intellectual property rights, we could be required to pay damages, potentially including treble damages, if we are found to have willfully infringed. In addition, we or our licensors, or any current or future strategic partners, may choose to seek, or be required to seek, a license from a third party, which may not be available on acceptable terms, if at all. Even if a license can be obtained on acceptable terms, the rights may be non-exclusive, which could give our competitors access to the same technology or intellectual property rights licensed to us. If we fail to obtain a required license, we or any current or future collaborator may be unable to effectively market therapeutic candidates based on our technology, which could limit our ability to generate revenue or achieve profitability and possibly prevent us from generating revenue sufficient to sustain our operations. In addition, we may find it necessary to pursue claims or initiate lawsuits to protect or enforce our patent or other intellectual property rights. The cost to us in defending or initiating any litigation or other proceeding relating to patent or other proprietary rights, even if resolved in our favor, could be substantial, and litigation would divert our management's attention. Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could delay our research and development efforts and limit our ability to continue our operations.

If we were to initiate legal proceedings against a third party to enforce a patent covering one of our therapeutics or our technology, the defendant could counterclaim that our patent is invalid or unenforceable. In patent litigation in the U.S., defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, for example, lack of novelty, obviousness or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. The outcome following legal assertions of invalidity and unenforceability during patent litigation is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of the patent protection on one or more of our therapeutics or certain aspects of our technology. Such a loss of patent protection could have a material adverse impact on our business. Patents and other intellectual property rights also will not protect our technology if competitors design around our protected technology without legally infringing our patents or other intellectual property rights.

It is also possible that we have failed to identify relevant third party patents or applications. For example, U.S. applications filed before November 29, 2000 and certain U.S. applications filed after that date that will not be filed outside the U.S. remain confidential until patents issue. Patent applications in the U.S. and elsewhere are published approximately 18 months after the earliest filing for which priority is claimed, with such earliest filing date being commonly referred to as the priority date. Therefore, patent applications covering our therapeutics or technology could have been filed by others without our knowledge. Additionally, pending patent applications which have been published can, subject to certain limitations, be later amended in a manner that could cover our SNA technology, our therapeutics or the use of our therapeutics. Third party intellectual property right holders may also actively bring infringement claims against us. We cannot guarantee that we will be able to successfully settle or otherwise resolve such infringement claims. If we are unable to successfully settle future claims on terms acceptable to us, we may be required to engage in or continue costly, unpredictable and time-consuming litigation and may be prevented from or experience substantial delays in marketing our therapeutics. If we fail in any such dispute, in addition to being forced to pay damages, we may be temporarily or permanently prohibited from commercializing any of our therapeutic candidates that are held to be infringing. We might, if possible, also be forced to redesign therapeutic candidates so that we no longer infringe the third party intellectual property rights. Any of these events, even if we were ultimately to prevail, could require us to divert substantial financial and management resources that we would otherwise be able to devote to our business.

If we fail to comply with our obligations under any license, collaboration or other agreements, we may be required to pay damages and could lose intellectual property rights that are necessary for developing and protecting our therapeutic candidates or we could lose certain rights to grant sublicenses.

Our current licenses impose, and any future licenses we enter into are likely to impose, various development, commercialization, funding, milestone, royalty, diligence, sublicensing, insurance, patent prosecution and enforcement, and other obligations on us. If we breach any of these obligations, or use the intellectual property licensed to us in an unauthorized manner, we may be required to pay damages and the licensor may have the right to terminate the license, which could result in us being unable to develop, manufacture and sell therapeutics that are covered by the licensed technology or could enable a competitor to gain access to the licensed technology. Moreover, our licensors may own or control intellectual property that has not been licensed to us and, as a result, we may be subject to claims, regardless of their merit, that we are infringing or otherwise violating the licensor's rights in such unlicensed intellectual property. In addition, while we cannot currently determine the amount of the royalty obligations we would be required to pay on sales of future therapeutics, if any, the amounts may be significant. The amount of our future royalty obligations will depend on the technology and intellectual property we use in therapeutics that we successfully develop and commercialize, if any. Therefore, even if we successfully develop and commercialize therapeutics, we may be unable to achieve or maintain profitability.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patent protection for certain aspects of our therapeutic candidates, we also consider trade secrets, including confidential and unpatented know-how important to the maintenance of our competitive position. We protect trade secrets and confidential and unpatented know-how, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to such knowledge, such as our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants that obligate them to maintain confidentiality and assign their inventions to us. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts in the U.S. and certain foreign jurisdictions are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

Under the terms of the Northwestern University License Agreements, NU could publish research findings relating to the patent rights licensed to us by NU, which could have a material adverse effect on our business.

We are also subject both in the U.S. and outside the U.S. to various regulatory schemes regarding requests for the information we provide to regulatory authorities, which may include, in whole or in part, trade secrets or confidential commercial information. While we are likely to be notified in advance of any disclosure of such information and would likely object to such disclosure, there can be no assurance that our challenge to the request would be successful.

We may be subject to claims that we or our employees or consultants have wrongfully used or disclosed alleged trade secrets of our employees' or consultants' former employers or their clients. These claims may be costly to defend and if we do not successfully do so, we may be required to pay monetary damages and may lose valuable intellectual property rights or personnel.

Many of our employees were previously employed at universities or pharmaceutical or biotechnology companies, including our competitors or potential competitors. Although no claims against us are currently pending, we may be subject to claims that these employees or we have inadvertently or otherwise used or disclosed trade secrets or other proprietary information of their former employers. Litigation may be necessary to defend against these claims. If we fail in defending such claims, in addition to paying monetary damages, we may lose valuable

intellectual property rights or personnel. A loss of key research personnel or their work product could hamper our ability to commercialize, or prevent us from commercializing, our therapeutic candidates, which could severely harm our business. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to management.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

Our trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names or may be forced to stop using these names, which we need for name recognition by potential partners or customers in our markets of interest. If we are unable to establish name recognition based on our trademarks and trade names, we may not be able to compete effectively and our business may be adversely affected.

Third parties may independently develop similar or superior technology.

There can be no assurance that others will not independently develop, or have not already developed, similar or more advanced technologies than our technology; or that others will not design around, or have not already designed around, aspects of our technology and/or our trade secrets developed therefrom. If third parties develop technology similar or superior to our technology, or they successfully design around our current or future technology, our competitive position, business prospects, and results of operations could be materially and adversely affected.

The intellectual property which we have licensed from Northwestern University was discovered through government funded programs and thus may be subject to federal regulations such as "march-in" rights, certain reporting requirements, and a preference for U.S. industry. Compliance with such regulations may limit our exclusive rights, subject us to expenditure of resources with respect to reporting requirements, and limit our ability to contract with non-U.S. manufacturers.

We have licensed certain intellectual property from NU pursuant to the Northwestern University License Agreements. The Northwestern University License Agreements indicate that the rights licensed to us by NU are subject to the obligations to and the rights of the U.S. government, including those set forth in the Bayh-Dole Act of 1980 (the "Bayh-Dole Act"). As a result, the U.S. government may have certain rights to intellectual property embodied in our current or future therapeutics based on the licensed NU intellectual property. These U.S. government rights in certain inventions developed under a government-funded program include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the U.S. government has the right to require us to grant exclusive, partially exclusive, or non-exclusive licenses to any of these inventions to a third party if it determines that: (i) adequate steps have not been taken to commercialize the invention; (ii) government action is necessary to meet public health or safety needs; or (iii) government action is necessary to meet requirements for public use under federal regulations, also referred to as "march-in rights." While the U.S. government has sparingly used, and to the Company's knowledge never successfully exercised, such march-in rights, any exercise of the march-in rights by the U.S. government could harm our competitive position, business, financial condition, results of operations, and prospects. If the U.S. government exercises such march-in rights, we may receive compensation that is deemed reasonable by the U.S. government in its sole discretion, which may be less than what we might be able to obtain in the open market. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us to expend substantial resources.

In addition, the U.S. government requires that any therapeutics embodying any invention generated through the use of U.S. government funding be manufactured substantially in the U.S. The manufacturing preference requirement can be waived if the owner of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to manufacture substantially in the United States or that under the circumstances domestic manufacture is not commercially feasible. This preference for U.S. manufacturers may limit our ability to contract with non-U.S. therapeutic manufacturers for therapeutics covered by such intellectual property.

Risks Related to Government Regulation

We may be unable to obtain U.S. or foreign regulatory approval and, as a result, unable to commercialize our therapeutic candidates.

Our therapeutic candidates are subject to extensive governmental regulations relating to, among other things, research, testing, development, manufacturing, safety, efficacy, approval, recordkeeping, reporting, labeling, storage, packaging, advertising and promotion, pricing, marketing, sampling, and distribution of therapeutics. Rigorous preclinical testing and clinical trials and an extensive regulatory approval process are required to be successfully completed in the U.S. and in many foreign jurisdictions before a new therapeutic can be marketed. Satisfaction of these and other regulatory requirements is costly, time consuming, uncertain and subject to unanticipated delays. It is possible that none of the therapeutic candidates we may develop will obtain the regulatory approvals necessary for us or any current or future collaborators to begin selling them.

We have very limited experience in conducting and managing the clinical trials necessary to obtain regulatory approvals, including approval by the FDA as well as foreign regulatory authorities, such as the EMA and European Union national competent authorities. The time required to obtain FDA and foreign regulatory approvals is unpredictable but typically takes many years following the commencement of clinical trials, depending upon the type, complexity and novelty of the therapeutic candidate. The standards that the FDA and its foreign counterparts use when regulating us are not always applied predictably or uniformly and can change. Any analysis we perform of data from preclinical and clinical activities is subject to confirmation and interpretation by regulatory authorities, which could delay, limit or prevent regulatory approval. We may also encounter unexpected delays or increased costs due to new government regulations, for example, from future legislation or administrative action, or from changes in the policy of the FDA or foreign regulatory authorities during the period of therapeutic development, clinical trials and regulatory review by the FDA or foreign regulatory authorities. It is impossible to predict whether legislative changes will be enacted, or whether FDA or foreign laws, regulations, guidance or interpretations will be changed, or what the impact of such changes, if any, may be.

Because the therapeutics we are developing may represent a new class of therapeutic, the FDA and its foreign counterparts have not yet established any definitive policies, practices or guidelines in relation to these therapeutics. While we believe the therapeutic candidates that we are currently developing are regulated as new drugs under the Federal Food, Drug, and Cosmetic Act of 1938 ("FDCA"), the FDA could decide to regulate them or other therapeutics we may develop as biologics under the Public Health Service Act. The lack of policies, practices or guidelines may hinder or slow review by the FDA or foreign regulatory authorities of any regulatory filings that we may submit. Moreover, the FDA may respond to these submissions by defining requirements we may not have anticipated. Such responses could lead to significant delays in the clinical development of our therapeutic candidates. In addition, because there may be therapeutic candidates approved for some of the diseases for which we may seek approval, in order to receive regulatory approval, we may need to demonstrate through clinical trials that the therapeutic candidates we develop to treat these diseases, if any, are not only safe and effective, but safer or more effective than existing products.

Any delay or failure in obtaining required approvals could have a material adverse effect on our ability to generate revenues from the particular therapeutic candidate for which we are seeking approval. Furthermore, any regulatory approval to market a therapeutic may be subject to limitations on the approved uses for which we may market the therapeutic or the labeling or other restrictions. Regulatory authorities also may impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the therapeutic. In addition, the FDA has the authority to require a REMS plan as part of a NDA or Biologics License Application ("BLA"), or after approval, which may impose further requirements or restrictions on the distribution or use of an approved drug or biologic, such as limiting prescribing to certain physicians or medical centers that have undergone specialized training, limiting treatment to patients who meet certain safe-use criteria and requiring treated patients to enroll in a registry. These limitations and restrictions may limit the size of the market for the therapeutic and affect coverage and reimbursement by third party payors.

We are also subject to numerous foreign regulatory requirements governing, among other things, the conduct of clinical trials, manufacturing and marketing authorization, pricing and third party reimbursement. The foreign

regulatory approval process varies among countries and may include all of the risks associated with FDA approval described above as well as risks attributable to the satisfaction of local regulations in foreign jurisdictions. Moreover, the time required to obtain approval may differ from that required to obtain FDA approval. Approval by the FDA does not ensure approval by regulatory authorities outside the U.S. and vice versa.

If we or current or future collaborators, manufacturers or service providers fail to comply with healthcare laws and regulations, we or they could be subject to enforcement actions, which could affect our ability to develop, market and sell our therapeutics and may harm our reputation.

Although we do not currently have any products on the market, once we begin commercializing our therapeutic candidates, we will be subject to additional healthcare statutory and regulatory requirements and enforcement by the federal, state and foreign governments of the jurisdictions in which we conduct our business. Healthcare providers, physicians and third-party payors play a primary role in the recommendation and prescription of any therapeutic candidates for which we obtain marketing approval. Our future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute our therapeutic candidates for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations, include the following:

- the U.S. federal Anti-Kickback Statute, which prohibits, among other things, persons from soliciting, receiving, offering or providing remuneration, directly or indirectly, to induce either the referral of an individual for a healthcare item or service, or the purchasing or ordering of an item or service, for which payment may be made, in whole or in part, under a federal healthcare program such as Medicare or Medicaid;
- the U.S. federal False Claims Act, which imposes criminal and civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government. In addition, the government may assert that a claim including items and services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act;
- HIPAA, All Payor Fraud Law, which imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services; similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- HIPAA, as amended by HITECH, and its implementing regulations, which impose obligations on certain covered entity healthcare providers, health plans, and healthcare clearinghouses as well as their business associates that perform certain services involving the use or disclosure of individually identifiable health information, including mandatory contractual terms, with respect to safeguarding the privacy, security, and transmission of individually identifiable health information, and require notification to affected individuals and regulatory authorities of certain breaches of security of individually identifiable health information;
- the federal Physician Payment Sunshine Act and the implementing regulations, also referred to as "Open Payments," issued under the ACA, which require that manufacturers of pharmaceutical and biological drugs reimbursable under Medicare, Medicaid, and Children's Health Insurance Programs report to the Department of Health and Human Services all consulting fees, travel reimbursements, research grants, and other payments, transfers of value or gifts made to physicians and teaching hospitals with limited exceptions; and
- analogous state laws and regulations, such as, state anti-kickback and false claims laws potentially applicable to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; and some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines

and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other healthcare providers or marketing expenditures, and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Ensuring that our future business arrangements with third-parties comply with applicable healthcare laws and regulations could involve substantial costs. If our operations are found to be in violation of any such requirements, we may be subject to penalties, including civil or criminal penalties, monetary damages, the curtailment or restructuring of our operations, or exclusion from participation in government contracting, healthcare reimbursement or other government programs, including Medicare and Medicaid, any of which could adversely affect our financial results. Although effective compliance programs can mitigate the risk of investigation and prosecution for violations of these laws, these risks cannot be entirely eliminated. Any action against us for an alleged or suspected violation could cause us to incur significant legal expenses and could divert our management's attention from the operation of our business, even if our defense is successful. In addition, achieving and sustaining compliance with applicable laws and regulations may be costly to us in terms of money, time and resources.

If we or current or future collaborators, manufacturers or service providers fail to comply with applicable federal, state or foreign laws or regulations, we could be subject to enforcement actions, which could affect our ability to develop, market and sell our therapeutics successfully and could harm our reputation and lead to reduced acceptance of our therapeutics by the market. These enforcement actions include, among others:

- adverse regulatory inspection findings;
- warning or untitled letters;
- voluntary product recalls or public notification or medical product safety alerts to healthcare professionals;
- restrictions on, or prohibitions against, marketing our therapeutics;
- restrictions on, or prohibitions against, importation or exportation of our therapeutics;
- suspension of review or refusal to approve pending applications or supplements to approved applications;
- exclusion from participation in government-funded healthcare programs;
- exclusion from eligibility for the award of government contracts for our therapeutics;
- FDA debarment;
- suspension or withdrawal of therapeutic approvals;
- seizures or administrative detention of therapeutics;
- injunctions; and
- civil and criminal penalties and fines.

Any therapeutics we develop may become subject to unfavorable pricing regulations, third party coverage and reimbursement practices or healthcare reform initiatives, thereby harming our business.

The regulations that govern marketing approvals, pricing and reimbursement for new therapeutics vary widely from country to country. Some countries require approval of the sale price of a therapeutic before it can be marketed. In many countries, the pricing review period begins after marketing or therapeutic licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. Although we intend to monitor these regulations, our programs are currently in the early stages of development and we will not be able to assess the impact of price regulations for a number of years. As a result, we might obtain regulatory approval for a therapeutic in a particular country, but then be subject to price

regulations that delay our commercial launch of the therapeutic and negatively impact the revenues we are able to generate from the sale of the therapeutic in that country.

Our ability to commercialize any therapeutics successfully also will depend in part on the extent to which coverage and reimbursement for these therapeutics and related treatments will be available from government health administration authorities, private health insurers and other organizations. However, there may be significant delays in obtaining coverage for newly-approved therapeutics. Moreover, eligibility for coverage does not necessarily signify that a therapeutic will be reimbursed in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution costs. Also, interim payments for new therapeutics, if applicable, may be insufficient to cover our costs and may not be made permanent. Thus, even if we succeed in bringing one or more therapeutics to the market, these therapeutics may not be considered cost-effective, and the amount reimbursed for any therapeutics may be insufficient to allow us to sell our therapeutics on a competitive basis. Because our programs are in the early stages of development, we are unable at this time to determine their cost effectiveness or the likely level or method of reimbursement. Increasingly, the third party payors who reimburse patients or healthcare providers, such as government and private insurance plans, are seeking greater upfront discounts, additional rebates and other concessions to reduce the prices for therapeutics. If the price we are able to charge for any therapeutics we develop, or the reimbursement provided for such therapeutics, is inadequate in light of our development and other costs, our return on investment could be adversely affected.

We currently expect that some therapeutics we develop may need to be administered under the supervision of a physician on an outpatient basis. Under currently applicable U.S. law, certain therapeutics that are not usually self-administered (including injectable therapeutics) may be eligible for coverage under Medicare through Medicare Part B. Medicare Part B is part of original Medicare, the federal health care program that provides health care benefits to the aged and disabled, and covers outpatient services and supplies, including certain pharmaceutical products that are medically necessary to treat a beneficiary's health condition. Specifically, Medicare Part B coverage may be available for eligible beneficiaries when the following, among other requirements, have been satisfied:

- the product is reasonable and necessary for the diagnosis or treatment of the illness or injury for which the product is administered according to accepted standards of medical practice;
- the product is typically furnished incident to a physician's services;
- the indication for which the product will be used is included or approved for inclusion in certain Medicare-designated pharmaceutical compendia (when used for an off-label use); and
- the product has been approved by the FDA.

Under current law, as a condition of receiving Medicare Part B reimbursement (the Medicare program that generally covers physician-administered, outpatient drugs) for a manufacturer's eligible drugs or biologicals, the manufacturer is required to participate in other government healthcare programs, including the Medicaid Drug Rebate Program, the Veterans Health Administration program, and the 340B Drug Pricing Program. The Medicaid Drug Rebate Program requires pharmaceutical manufacturers to enter into and have in effect a national rebate agreement with the Secretary of the Department of Health and Human Services as a condition for states to receive federal matching funds for the manufacturer's outpatient drugs furnished to Medicaid patients. Under the 340B Drug Pricing Program, the manufacturer must extend discounts to entities eligible to participate in the program. Average prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of therapeutics from countries where they may be sold at lower prices than in the U.S. Reimbursement rates under Medicare Part B would depend in part on whether the newly approved therapeutic would be eligible for a unique billing code. Self-administered therapeutics are typically reimbursed under Medicare Part D, and therapeutics that are administered in an inpatient hospital setting are typically reimbursed under Medicare Part A under a bundled payment. It is difficult for us to predict how Medicare coverage and reimbursement policies will be applied to our therapeutics in the future and coverage and reimbursement under different federal healthcare programs are not always consistent. Medicare reimbursement rates may also reflect budgetary constraints placed on the Medicare program.

Third-party payors often rely upon Medicare coverage policies and payment limitations in setting their own reimbursement rates. These coverage policies and limitations may rely, in part, on compendia listings for approved therapeutics. Our inability to promptly obtain relevant compendia listings, coverage, and adequate reimbursement from both government-funded and private payors for new therapeutics we develop and for which we obtain regulatory approval could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our financial condition.

We believe that the efforts of governments and third party payors to contain or reduce the cost of healthcare and legislative and regulatory proposals to broaden the availability of healthcare will continue to affect the business and financial condition of pharmaceutical and biotechnology companies. A number of legislative and regulatory changes in the healthcare system in the U.S. and other major healthcare markets have been proposed, and such efforts have expanded substantially in recent years. These developments could, directly or indirectly, affect our ability to sell our therapeutics, if approved, at a favorable price.

For example, in the U.S., in 2010, the U.S. Congress passed the Affordable Care Act, as amended by the Health Care and Education Reconciliation Act (collectively, the "ACA"), a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of health spending, enhance remedies against fraud and abuse, add new transparency requirements for the healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional policy reforms.

Among the provisions of the ACA addressing coverage and reimbursement of pharmaceutical products, of importance to our potential therapeutic candidates are the following:

- Increases to pharmaceutical manufacturer rebate liability under the Medicaid Drug Rebate Program due to an increase in the minimum basic Medicaid rebate on most branded prescription drugs and the application of Medicaid rebate liability to drugs used in risk-based Medicaid managed care plans.
- The expansion of the 340B Drug Pricing Program to require discounts for "covered outpatient drugs" sold to certain children's hospitals, critical access hospitals, freestanding cancer hospitals, rural referral centers, and sole community hospitals.
- Requirements imposed on pharmaceutical companies to offer discounts on brand-name drugs to patients who fall within the Medicare Part D coverage gap, commonly referred to as the "Donut Hole."
- Requirements imposed on pharmaceutical companies to pay an annual non-tax-deductible fee to the federal government based on each company's market share of prior year total sales of branded drugs to certain federal healthcare programs, such as Medicare, Medicaid, Department of Veterans Affairs, and Department of Defense. Since we currently expect our branded pharmaceutical sales to constitute a small portion of the total federal healthcare program pharmaceutical market, we do not currently expect this annual assessment to have a material impact on our financial condition.
- For therapeutic candidates classified as biologics, marketing approval for a follow-on biologic therapeutic may not become effective until 12 years after the date on which the reference innovator biologic therapeutic was first licensed by the FDA, with a possible six-month extension for pediatric therapeutics. After this exclusivity ends, it may be possible for biosimilar manufacturers to enter the market, which is likely to reduce the pricing for such therapeutics and could affect our profitability if our therapeutics are classified as biologics.

Separately, pursuant to the health reform legislation and related initiatives, the Centers for Medicare and Medicaid Services ("CMS") is working with various healthcare providers to develop, refine, and implement Accountable Care Organizations ("ACOs"), and other innovative models of care for Medicare and Medicaid beneficiaries, including the Bundled Payments for Care Improvement Initiative, the Comprehensive Primary Care Initiative, the Duals Demonstration, and other models. The continued development and expansion of ACOs and other innovative models of care will have an uncertain impact on any future reimbursement we may receive for approved therapeutics administered by such organizations.

In addition, in recent years, the U.S. Congress has enacted various laws seeking to reduce the federal debt level and contain healthcare expenditures. For example, as a result of the Budget Control Act of 2011 and the Bipartisan Budget Act of 2015, an annual 2% reduction to Medicare payments took effect on April 1, 2013 and has been extended through 2025. These across-the-board spending cuts could adversely affect our future revenues, earnings, and cash flows.

The financial impact of U.S. healthcare reform legislation over the next few years will depend on a number of factors, including the policies reflected in implementing regulations and guidance and changes in sales volumes for therapeutics affected by the legislation.

From time to time, legislation is drafted, introduced and passed in the U.S. Congress that could significantly change the statutory provisions governing coverage, reimbursement, and marketing of products regulated by CMS or other government agencies. In addition to new legislation, CMS coverage and reimbursement policies are often revised or interpreted in ways that may significantly affect our business and our products. In particular, we expect that the new administration and the U.S. Congress will seek to modify, repeal, or otherwise invalidate all, or certain provisions of, the U.S. healthcare reform legislation. Since taking office, President Trump has continued to support the repeal of all or portions of the ACA. President Trump has also issued an executive order in which he stated that it is his administration's policy to seek the prompt repeal of the ACA and in which he directed executive departments and federal agencies to waive, defer, grant exemptions from, or delay the implementation of the provisions of the ACA to the maximum extent permitted by law. There is still uncertainty with respect to the impact President Trump's Administration and the U.S. Congress may have, if any, and any changes will likely take time to unfold. Such reforms could have an adverse effect on anticipated revenues from therapeutic candidates that we may successfully develop and for which we may obtain regulatory approval and may affect our overall financial condition and ability to develop therapeutic candidates. However, we cannot predict the ultimate content, timing or effect of any healthcare reform legislation or the impact of potential legislation on us.

The healthcare industry is heavily regulated in the U.S. at the federal, state, and local levels, and our failure to comply with applicable requirements may subject us to penalties and negatively affect our financial condition.

As a healthcare company, our operations, clinical trial activities and interactions with healthcare providers may be subject to extensive regulation in the U.S., particularly if the company receives FDA approval for any of its therapeutics in the future. For example, if we receive FDA approval for a therapeutic for which reimbursement is available under a federal healthcare program (e.g., Medicare, Medicaid), it would be subject to a variety of federal laws and regulations, including those that prohibit the filing of false or improper claims for payment by federal healthcare programs (e.g., the federal False Claims Act), prohibit unlawful inducements for the referral of business reimbursable by federal healthcare programs (e.g., the federal Anti-Kickback Statute), and require disclosure of certain payments or other transfers of value made to U.S.-licensed physicians and teaching hospitals, or Open Payments. We are not able to predict how third parties will interpret these laws and apply applicable governmental guidance and may challenge our practices and activities under one or more of these laws. If our past or present operations are found to be in violation of any of these laws, we could be subject to civil and criminal penalties, which could hurt our business, our operations and financial condition.

Similarly, HIPAA prohibits, among other offenses, knowingly and willfully executing a scheme to defraud any health care benefit program, including private payors, or falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for items or services under a health care benefit program. To the extent that we act as a business associate to a healthcare provider engaging in electronic transactions, we may also be subject to the privacy and security provisions of HIPAA, as amended by HITECH, which restricts the use and disclosure of patient-identifiable health information, mandates the adoption of standards relating to the privacy and security of patient-identifiable health information, and requires the reporting of certain security breaches to healthcare provider customers with respect to such information. Additionally, many states have enacted similar laws that may impose more stringent requirements on entities like ours. Failure to comply with applicable laws and regulations could result in substantial penalties and adversely affect our financial condition and results of operations.

Our ability to obtain services, reimbursement or funding from the federal government may be impacted by possible reductions in federal spending.

U.S. federal government agencies currently face potentially significant spending reductions. The Budget Control Act of 2011 (the "BCA") established a Joint Select Committee on Deficit Reduction, which was tasked with achieving a reduction in the federal debt level of at least $1.2 trillion. That committee did not draft a proposal by the BCA's deadline. As a result, automatic cuts, referred to as sequestration, in various federal programs were scheduled to take place, beginning in January 2013, although the American Taxpayer Relief Act of 2012 delayed the BCA's automatic cuts until March 1, 2013. While the Medicare program's eligibility and scope of benefits are generally exempt from these cuts, Medicare payments to providers and Part D health plans are not exempt. The BCA did, however, provide that the Medicare cuts to providers and Part D health plans would not exceed two percent. President Obama issued the sequestration order on March 1, 2013, and cuts went into effect on April 1, 2013. Additionally, the Bipartisan Budget Act of 2015 extended sequestration for Medicare through fiscal year 2025.

The U.S. federal budget remains in flux, which could, among other things, cut Medicare payments to providers. The Medicare program is frequently mentioned as a target for spending cuts. The full impact on our business of any future cuts in Medicare or other programs is uncertain. In addition, we cannot predict any impact President Trump's administration and the U.S. Congress may have on the federal budget. If federal spending is reduced, anticipated budgetary shortfalls may also impact the ability of relevant agencies, such as the FDA or the National Institutes of Health, to continue to function at current levels. Amounts allocated to federal grants and contracts may be reduced or eliminated. These reductions may also impact the ability of relevant agencies to timely review and approve therapeutic research and development, manufacturing, and marketing activities, which may delay our ability to develop, market, and sell any therapeutics we may develop.

If any of our therapeutic candidates receives marketing approval and we or others later identify undesirable side effects caused by the therapeutic candidate, our ability to market and derive revenue from the therapeutic candidates could be compromised.

In the event that any of our therapeutic candidates receive regulatory approval and we or others identify undesirable side effects, adverse events or other problems caused by one of our therapeutics, any of the following adverse events could occur, which could result in the loss of significant revenue to us and materially and adversely affect our results of operations and business:

- regulatory authorities may withdraw their approval of the therapeutic or seize the therapeutic;
- we may need to recall the therapeutic or change the way the therapeutic is administered to patients;
- additional restrictions may be imposed on the marketing of the particular therapeutic or the manufacturing processes for the therapeutic or any component thereof;
- we may be subject to fines, restitution or disgorgement of profits or revenues, injunctions, or the imposition of civil penalties or criminal prosecution;
- regulatory authorities may require the addition of labeling statements, such as a "black box" warning or a contraindication;
- regulatory authorities may require us to implement a REMS, or to conduct post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the therapeutic;
- we may be required to create a Medication Guide outlining the risks of such side effects for distribution to patients;
- we could be sued and held liable for harm caused to patients;
- the therapeutic may become less competitive; and
- our reputation may suffer.

Significant developments stemming from the United Kingdom's recent referendum on membership in the EU could have a material adverse effect on our business.

On June 23, 2016, the United Kingdom held a referendum and voted in favor of leaving the European Union ("EU"). This referendum has created political and economic uncertainty, particularly in the United Kingdom and the EU, and this uncertainty may last for years. Any business we conduct, now and in the future, in the United Kingdom, the EU, and worldwide could be affected during this period of uncertainty, and perhaps longer, by the impact of the United Kingdom's referendum. The referendum, and the likely withdrawal of the United Kingdom from the EU it triggers, has caused and, along with events potentially occurring in the future as a consequence of the United Kingdom's withdrawal, including the possible breakup of the United Kingdom, may continue to cause significant volatility in global financial markets, including in global currency and debt markets. This volatility could cause a slowdown in economic activity in the United Kingdom, Europe, or globally, which could adversely affect our operating results and growth prospects. In addition, our business could be negatively affected by new trade agreements between the United Kingdom and other countries, including the U.S., and by the possible imposition of trade or other regulatory barriers in the United Kingdom.

It is currently unclear how regulations affecting clinical trials, the approval of our future therapeutic candidates, and the sale of these therapeutic candidates will be affected by this referendum either in the United Kingdom or elsewhere in Europe. These possible negative impacts, and others resulting from the United Kingdom's actual or threatened withdrawal from the EU, may adversely affect our operating results and growth prospects.

Risks Related to Our Common Stock and the Offering

We are an "emerging growth company" and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies will make our common stock less attractive to investors.

We are an "emerging growth company" as defined in the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including (1) not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, (2) reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements and (3) exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. In addition, as an emerging growth company, we are only required to provide two years of audited financial statements and two years of selected financial data. We could be an emerging growth company for up to five years, although circumstances could cause us to lose that status earlier, including if the market value of our common stock held by non-affiliates exceeds $700.0 million as of any June 30 before that time or if we have total annual gross revenue of $1.1 billion or more during any fiscal year before that time, in which cases we would no longer be an emerging growth company as of the following December 31, or if we issue more than $1.0 billion in non-convertible debt during any three-year period before that time, in which case we would no longer be an emerging growth company immediately. Even after we no longer qualify as an emerging growth company, we may still qualify as a "smaller reporting company" which would allow us to take advantage of many of the same exemptions from disclosure requirements including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our share price may be more volatile.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

Our stock price may be volatile and purchasers of our common stock could incur substantial losses.

If a market for our common stock develops, its market price could fluctuate substantially due to a variety of factors, including the other risks described in this section of the Quarterly Report on Form 10-Q titled "Risk Factors" and the following:

- the success of competitive therapeutics or technologies;
- results of our preclinical studies and clinical trials of our therapeutic candidates, or those of our competitors, or any current or future collaborators;
- regulatory or legal developments in the U.S. and other countries, especially changes in laws or regulations applicable to our therapeutics;
- introductions and announcements of new therapeutics by us, our future commercialization partners, or our competitors, and the timing of these introductions or announcements;
- actions taken by regulatory agencies with respect to our therapeutics, clinical studies, manufacturing process or sales and marketing terms;
- actual or anticipated variations in our financial results or those of companies that are perceived to be similar to us;
- the success of our efforts to acquire or in-license additional technologies, therapeutics or therapeutic candidates;
- developments concerning any current or future collaborations, including but not limited to those with our sources of manufacturing supply and our commercialization partners;
- announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures or capital commitments;
- developments or disputes concerning patents or other proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our therapeutics;
- our ability or inability to raise additional capital and the terms on which we raise it;
- the recruitment or departure of key personnel;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- actual or anticipated changes in earnings estimates or changes in stock market analyst recommendations regarding our common stock, other comparable companies or our industry generally;
- our failure or the failure of our competitors to meet analysts' projections or guidance that we or our competitors may give to the market;
- fluctuations in the valuation of companies perceived by investors to be comparable to us;
- announcement and expectation of additional financing efforts;
- speculation in the press or investment community;
- trading volume of our common stock;
- sales of our common stock by us or our stockholders;
- the concentrated ownership of our common stock;

- changes in accounting principles;
- terrorist acts, acts of war or periods of widespread civil unrest;
- natural disasters and other calamities; and
- general economic, industry and market conditions.

In addition, the stock markets in general, and the markets for pharmaceutical and biotechnology stocks in particular, have experienced extreme volatility that has been often unrelated to the operating performance of the issuer. These broad market and industry factors may seriously harm the market price of our common stock, regardless of our operating performance.

The future issuance of equity or of debt securities that are convertible into equity may dilute your investment and reduce your equity interest.

We may choose to raise additional capital in the future, depending on market conditions, strategic considerations and operational requirements. To the extent that additional capital is raised through the issuance of shares or other securities convertible into shares, our stockholders will be diluted. Future issuances of our common stock or other equity securities, or the perception that such sales may occur, could adversely affect the prevailing market price of our common stock and impair our ability to raise capital through future offerings of equity or equity-linked securities. For example, we have agreed, at our expense, to prepare a registration statement, and to cause us to file a registration statement with the SEC registering the resale of up to 46,269,418 shares of our common stock issued in connection with the Merger and the Offering. Once effective, the registration statement will permit the resale of these shares at any time for up to five years following the effective date of such registration statement. The resale of a substantial number of shares of our common stock in the public market could adversely affect the market price for our common stock and make it more difficult for you to sell shares of our common stock at times and prices that you feel are appropriate. Furthermore, we expect that, because there will be a large number of shares registered pursuant to a registration statement, selling stockholders will continue to offer shares covered by such registration statement for a significant period of time, the precise duration of which cannot be predicted. Accordingly, the adverse market and price pressures resulting from an offering pursuant to a registration statement may continue for an extended period of time and continued negative pressure on the market price of our common stock could have a material adverse effect on our ability to raise additional equity capital.

Our debt obligations expose us to risks that could adversely affect our business, operating results and financial condition and may result in further dilution to our stockholders.

We have entered into a loan and security agreement with Hercules pursuant to which we may borrow in an aggregate principal amount of up to $10 million from Hercules at a floating per annum interest rate (based on a year consisting of 360 days) equal to the greater of either (i) 9.95% or (ii) the sum of (a) 9.95% plus (b) the prime rate (as reported in *The Wall Street Journal*) minus 3.50%. We must make interest only payments on the amounts borrowed until June 2017. Commencing on July 1, 2017, the loan began amortizing in equal monthly installments of principal and interest in an amount sufficient to fully amortize the outstanding principal balance of the loan over the remaining scheduled monthly payments due prior to the maturity date on September 1, 2019. Any remaining obligations under the loan agreement and other loan documents (other than the warrant) are due and payable on the maturity date. On the earliest to occur of the maturity date, the date we prepay the term loan in full or the date the loan otherwise becomes due and payable, we must pay the lender under the agreement an additional charge equal to 3.85% of the total amounts funded under the loan agreement. To the extent we desire to prepay the indebtedness prior to maturity, we will be obligated to pay a prepayment penalty to Hercules ranging from 1% to 3% of the amounts being prepaid, depending on when such prepayment occurs. The loan agreement was amended on October 10, 2016 to revise the language granting Hercules a contingent security interest in certain of our assets. Under the loan agreement, Hercules or its affiliates have a right to participate in a single subsequent unregistered financing by us in an amount of up $1.0 million on the same terms, conditions and pricing afforded to others participating in such financing. Hercules has not yet exercised this right to participate.

Our ability to make payments on this indebtedness depends on our ability to generate cash in the future. We expect to experience negative cash flow for the foreseeable future as we fund our operations and capital expenditures. There can be no assurance that we will be in a position to repay this indebtedness when due or obtain extensions of the maturity date. We anticipate that we will need to secure additional funding in order for us to be able to satisfy our obligations when due. We cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all. If that additional funding involves the sale of equity securities or convertible securities, it would result in the issuance of additional shares of our capital stock, which would result in dilution to our stockholders. The indebtedness is secured by substantially all of our assets other than intellectual property, on which we have given Hercules a negative pledge. In addition, under the loan agreement, we are subject to certain customary covenants that limit or restrict our ability to, among other things, incur additional indebtedness, grant any security interests, pay cash dividends, repurchase our common stock, make loans, or enter into certain transactions without the prior consent of Hercules.

This level of debt could have important consequences to you as an investor in our securities. For example, it could:

- limit our flexibility in planning for the development, clinical testing, approval and marketing of our products;
- place us at a competitive disadvantage compared to any of our competitors that are less leveraged than we are;
- increase our vulnerability to both general and industry-specific adverse economic conditions; and
- limit our ability to obtain additional funds.

The employment agreements with our executive officers may require us to pay severance benefits to officers in connection with termination of employment or upon a change of control of us, which could harm our financial condition.

Each of David A. Giljohann, our Chief Executive Officer, Ekambar Kandimalla, our Chief Scientific Officer, and David S. Snyder, our Chief Financial Officer, is entitled to receive cash severance equal to twelve months, six months, and six months, respectively, of his base salary if his employment is terminated by us without cause (as such term is defined in his employment offer letter). In addition, our 2015 Plan, which was assumed by us in the Merger, generally provides for accelerated vesting of equity awards upon the involuntary termination of an employee within the twelve month period following a change in control (as defined under the plan) and accelerated vesting of equity awards upon a change of control (as defined under the plan) for each of our executive officers. This vesting acceleration is intended to provide each of our executive officers with the full benefit of their equity awards and reward them for a successful outcome for our stockholders. The accelerated vesting of equity awards could result in dilution to our existing stockholders and harm the market price of our common stock. The payment of these severance benefits could harm our financial condition. In addition, these potential severance payments may discourage or prevent third parties from seeking a business combination with us.

There is currently no market for our common stock and we cannot assure you that any market will ever develop. You may therefore be unable to re-sell shares of our common stock at times and prices that you believe are appropriate.

Our common stock is not listed on a national securities exchange or any other exchange, or quoted on an over-the-counter market. Therefore, there is no trading market, active or otherwise, for our common stock and our common stock may never be included for trading on any stock exchange, automated quotation system or any over-the-counter market. Accordingly, our common stock is highly illiquid and you will likely experience difficulty in re-selling such shares at times and prices that you may desire and without depressing the market price for the shares or at all.

Our common stock may not be eligible for listing or quotation on any securities exchange.

We do not currently meet the initial quantitative listing standards of any national securities exchange or over-the-counter trading system. We cannot assure you that we will be able to meet the initial listing standards of any national securities exchange, or, if we do meet such initial listing standards, that we will be able to maintain any such listing. Further, the national securities exchanges are adopting so-called "seasoning" rules that will require that we meet certain requirements, including prescribed periods of time trading over-the-counter and minimum filings of periodic reports with the SEC, before we are eligible to apply for listing on such national securities exchanges. We intend to contact an authorized market maker for an over-the-counter quotation system for sponsorship of our common stock, but we cannot guarantee that such sponsorship will be approved and our common stock listed and quoted for sale. Even if our common stock is quoted for sale on an over-the-counter quotation system, buyers may be insufficient in numbers to allow for a robust market and it may prove impossible to sell your shares. In addition, an investor may find it difficult to obtain accurate quotations as to the market value of our common stock. In addition, if we fail to meet the criteria set forth in SEC regulations, various requirements would be imposed by law on broker-dealers who sell our securities to persons other than established customers and accredited investors. Consequently, such regulations may deter broker-dealers from recommending or selling our common stock, which may further affect its liquidity. This would also make it more difficult for us to raise additional capital.

The designation of our common stock as a "penny stock" would limit the liquidity of our common stock.

Our common stock may be deemed a "penny stock" (as that term is defined under Rule 3a51-1 of the Exchange Act) in any market that may develop in the future. Generally, a "penny stock" is a common stock that is not listed on a securities exchange and trades for less than $5.00 a share. Prices often are not available to buyers and sellers and the market may be very limited. Penny stocks in start-up companies are among the riskiest equity investments. Broker-dealers who sell penny stocks must provide purchasers of these stocks with a standardized risk-disclosure document prepared by the SEC. The document provides information about penny stocks and the nature and level of risks involved in investing in the penny stock market. A broker must also provide purchasers with bid and offer quotations and information regarding broker and salesperson compensation and make a written determination that the penny stock is a suitable investment for the purchaser and obtain the purchaser's written agreement to the purchase. Many brokers choose not to participate in penny stock transactions. Because of the penny stock rules, there may be less trading activity in penny stocks in any market that develops for our common stock in the future and stockholders are likely to have difficulty selling their shares.

FINRA sales practice requirements may limit a stockholder's ability to buy and sell our stock.

The Financial Industry Regulatory Authority ("FINRA") has adopted rules requiring that, in recommending an investment to a customer, a broker-dealer must have reasonable grounds for believing that the investment is suitable for that customer. Prior to recommending speculative or low-priced securities to their non-institutional customers, broker-dealers must make reasonable efforts to obtain information about the customer's financial status, tax status, investment objectives and other information. Under interpretations of these rules, FINRA has indicated its belief that there is a high probability that speculative or low-priced securities will not be suitable for at least some customers. If these FINRA requirements are applicable to us or our securities, they may make it more difficult for broker-dealers to recommend that at least some of their customers buy our common stock, which may limit the ability of our stockholders to buy and sell our common stock and could have an adverse effect on the market for and price of our common stock.

The shares of common stock issued in the Merger and the Offering are "restricted securities" and, as such, may not be sold except in limited circumstances.

None of the shares of common stock issued in the Merger and the Offering have been registered under the Securities Act or registered or qualified under any state securities laws. The shares of common stock issued in the Merger and the Offering were sold and/or issued and will be sold and/or issued pursuant to exemptions contained in and under those laws. Accordingly, such shares of common stock are "restricted securities" as defined in Rule 144 under the Securities Act and must, therefore, be held indefinitely unless registered under applicable federal and state securities laws, or an exemption is available from the registration requirements of those laws. The certificates representing the shares of common stock issued in the Merger and the Offering reflect their restricted status.

We have agreed to register the shares of common stock issued in the Merger and the Offering. We cannot assure you, however, that the SEC will declare the registration statement effective, thereby enabling the shares of common stock issued in the Merger or the Offering to be freely tradable. In addition, Rule 144 under the Securities Act, which permits the resale, subject to various terms and conditions, of limited amounts of restricted securities after they have been held for six months will not immediately apply to our common stock because we were at one time designated as a "shell company" under SEC regulations. Pursuant to Rule 144(i), securities issued by a current or former shell company that otherwise meet the holding period and other requirements of Rule 144 nevertheless cannot be sold in reliance on Rule 144 until one year after the date on which the issuer filed current "Form 10 information" (as defined in Rule 144(i)) with the SEC reflecting that it ceased being a shell company, and provided that at the time of a proposed sale pursuant to Rule 144, the issuer has satisfied certain reporting requirements under the Exchange Act. We believe this requirement to file Form 10 information has been satisfied by the filing of our Form 8-K.

Because, as a former shell company, the reporting requirements of Rule 144(i) will apply regardless of holding period, the restrictive legends on certificates for the shares of common stock issued in the Merger and the Offering cannot be removed except in connection with an actual sale that is subject to an effective registration statement under, or an applicable exemption from the registration requirements of, the Securities Act.

If we are unable to timely register the shares of common stock issued to stockholders in the Merger or the Offering, then the ability to re-sell shares of such common stock will be delayed.

We have agreed, at our expense, to prepare a registration statement, and to cause our Company to file a registration statement with the SEC registering the resale of up to 46,269,418 shares of our common stock issued in connection with the Merger and the Offering. To the extent such registration statement is not declared effective by the SEC, or there are delays resulting from the SEC review process and comments raised by the SEC during that process, the shares of common stock proposed to be covered by such registration statement will not be eligible for resale until the registration statement is effective or an exemption from registration, such as Rule 144, becomes available. If the registration statement is not filed within 60 days of the final closing of the Offering, then we may be subject to certain liquidated damages pursuant to the registration rights agreement we entered into with the holders of up to 46,269,418 shares of our common stock issued in connection with the Merger and the Offering.

Because our management will have broad discretion over the use of the net proceeds from the Offering, you may not agree with how we use them and the proceeds may not be invested successfully.

We intend to use the net proceeds to us from the Offering to fund research and development, preclinical studies and clinical trial expenses and potential in-licensing of intellectual property and technology or other acquisition activities, and other general corporate purposes, including funding the costs of operating as a public company, and therefore, our management will have broad discretion as to the use of the Offering proceeds. Accordingly, you will be relying on the judgment of our management with regard to the use of these net proceeds, and you will not have the opportunity, as part of your investment decision, to assess whether the proceeds are being used appropriately. It is possible that the proceeds will be invested in a way that does not yield a favorable, or any, return for our company.

If securities or industry analysts do not publish research or reports about our business, or if they issue an adverse or misleading opinion regarding our stock, our stock price and trading volume could decline.

The trading market for our common stock will be influenced by the research and reports that industry or securities analysts publish about us or our business. We do not currently have and may never obtain research coverage by securities and industry analysts. In addition, because we did not become a reporting company by conducting an underwritten initial public offering of our common stock, and because we will not be listed on a national securities exchange, security analysts of brokerage firms may not provide coverage of our Company. In addition, investment banks may be less likely to agree to underwrite secondary offerings on our behalf than they might if we became a public reporting company by means of an underwritten initial public offering, because they may be less familiar with our Company as a result of more limited coverage by analysts and the media, and because we became public at an early stage in our development. The failure to receive research coverage or support in the market for our shares will have an adverse effect on our ability to develop a liquid market for our common stock and the trading price for our stock would be negatively impacted.

In the event we obtain securities or industry analyst coverage, if any of the analysts who cover us issue an adverse or misleading opinion regarding us, our business model, our intellectual property or our stock performance, or if our target studies and operating results fail to meet the expectations of analysts, our stock price would likely decline. If one or more of these analysts cease coverage of us or fail to publish reports on us regularly, we could lose visibility in the financial markets, which in turn could cause our stock price or trading volume to decline.

Because the Merger was a reverse merger, the registration statement we file with respect to the shares of common stock received by investors in the Merger and the Offering may be subject to heightened scrutiny by the SEC.

Certain SEC rules are more restrictive when applied to reverse merger companies, such as the ability of stockholders to re-sell their shares of common stock pursuant to Rule 144, and the SEC may subject the registration statement we file with respect to the shares of common stock received by investors in the Merger and the Offering to heightened scrutiny. In addition, securities analysts of major brokerage firms may not provide coverage of our capital stock or business. Because we became a public reporting operating company through a reverse merger, there is no incentive to brokerage firms to recommend the purchase of our common stock. We cannot assure you that brokerage firms will want to provide analyst coverage of our capital stock or business in the future.

Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

Based on the beneficial ownership of our common stock as of September 30, 2017, our executive officers and directors, together with holders of five percent or more of our outstanding common stock and their respective affiliates, beneficially own approximately 70.5 percent of our outstanding common stock. As a result, these stockholders, if acting together, will continue to have significant influence over the outcome of corporate actions requiring stockholder approval, including the election of directors, any merger, consolidation or sale of all or substantially all of our assets and any other significant corporate transaction. The interests of these stockholders may not be the same as or may even conflict with your interests. For example, these stockholders could delay or prevent a change of control of our company, even if such a change of control would benefit our other stockholders, which could deprive our stockholders of an opportunity to receive a premium for their common stock as part of a sale of our company or our assets and might affect the prevailing market price of our common stock. The significant concentration of stock ownership may adversely affect the trading price of our common stock due to investors' perception that conflicts of interest may exist or arise.

Anti-takeover provisions in our charter documents and under the General Corporation Law of the State of Delaware could make an acquisition of us more difficult and may prevent attempts by our stockholders to replace or remove our management.

Provisions in our certificate of incorporation and our bylaws may delay or prevent an acquisition of us or a change in our management. These provisions include a classified board of directors, a prohibition on actions by written consent of the combined organization's stockholders, and the ability of the board of directors to issue preferred stock without stockholder approval. In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law ("DGCL"), which prohibits stockholders owning in excess of 15% of the outstanding combined organization voting stock from merging or combining with the combined organization. Although we believe these provisions collectively will provide for an opportunity to receive higher bids by requiring potential acquirers to negotiate with our board of directors, they would apply even if the offer may be considered beneficial by some stockholders. In addition, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove then-current management by making it more difficult for stockholders to replace members of the board of directors, which is responsible for appointing the members of management.

Anti-takeover provisions in our charter documents could discourage, delay or prevent a change in control of us and may affect the trading price of our common stock.

Our corporate documents and the DGCL contain provisions that may enable our board of directors to resist a change in control of us even if a change in control were to be considered favorable by our stockholders. These provisions:

- stagger the terms of our board of directors and require 66 and 2/3% stockholder voting to remove directors, who may only be removed for cause;
- authorize our board of directors to issue "blank check" preferred stock and to determine the rights and preferences of those shares, which may be senior to our common stock, without prior stockholder approval;
- establish advance notice requirements for nominating directors and proposing matters to be voted on by stockholders at stockholders' meetings;
- prohibit our stockholders from calling a special meeting and prohibit stockholders from acting by written consent;
- require 66 and 2/3% stockholder voting to effect certain amendments to our certificate of incorporation and bylaws; and
- prohibit cumulative voting in the election of directors, which limits the ability of minority stockholders to elect director candidates.

These provisions could discourage, delay or prevent a transaction involving a change in control of us. These provisions could also discourage proxy contests and make it more difficult for stockholders to elect directors of their choosing and cause us to take other corporate actions our stockholders desire.

We will incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives and corporate governance practices.

As a public company, and particularly after we are no longer an emerging growth company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of any national securities exchange or other exchange and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance, which in turn could make it more difficult for us to attract and retain qualified members of our board of directors. However, these rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

As a public company, we are required to furnish a report by our management on our internal control over financial reporting. However, while we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude, within the prescribed time frame or at all, that our internal control over financial reporting is effective as required by Section 404. If we identify one or more material weaknesses, it could result in an adverse reaction in the financial markets due to a loss of confidence in the

reliability of our financial statements. In addition, if we are not able to continue to meet these requirements, we may not be able to remain listed on any national securities exchange or other exchange or quoted on an over-the-counter market.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be your sole source of gain.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

We may incur significant costs from class action litigation due to our expected stock volatility.

Our stock price may fluctuate for many reasons, including as a result of public announcements regarding the progress of our development efforts or the development efforts of current or future collaborators or competitors, the addition or departure of our key personnel, variations in our quarterly operating results and changes in market valuations of pharmaceutical and biotechnology companies. This risk is especially relevant to us because pharmaceutical and biotechnology companies have experienced significant stock price volatility in recent years. When the market price of a stock has been volatile as our stock price may be, holders of that stock have occasionally brought securities class action litigation against the company that issued the stock. If any of our stockholders were to bring a lawsuit of this type against us, even if the lawsuit is without merit, we could incur substantial costs defending the lawsuit. The lawsuit could also divert the time and attention of our management.

Our amended and restated certificate of incorporation will designate the Court of Chancery of the State of Delaware as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, employees or agents.

Our amended and restated certificate of incorporation will provide that, unless we consent in writing to an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers, employees or agents to us or our stockholders, any action asserting a claim arising pursuant to any provision of the DGCL, our amended and restated certificate of incorporation or our amended and restated bylaws or any action asserting a claim that is governed by the internal affairs doctrine, in each case subject to the Court of Chancery having personal jurisdiction over the indispensable parties named as defendants therein and the claim not being one which is vested in the exclusive jurisdiction of a court or forum other than the Court of Chancery or for which the Court of Chancery does not have subject matter jurisdiction. Any person purchasing or otherwise acquiring any interest in any shares of our common stock shall be deemed to have notice of and to have consented to this provision of our amended and restated certificate of incorporation. This choice of forum provision may limit our stockholders' ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, employees or agents, which may discourage such lawsuits against us and our directors, officers, employees and agents even though an action, if successful, might benefit our stockholders. Stockholders who do bring a claim in the Court of Chancery could face additional litigation costs in pursuing any such claim, particularly if they do not reside in or near Delaware. The Court of Chancery may also reach different judgments or results than would other courts, including courts where a stockholder considering an action may be located or would otherwise choose to bring the action, and such judgments or results may be more favorable to us than to our stockholders. Alternatively, if a court were to find this provision of our amended and restated certificate of incorporation inapplicable to, or unenforceable in respect of, one or more of the specified types of actions or proceedings, we may incur additional costs associated with resolving such matters in other jurisdictions, which could have a material adverse effect on our business, financial condition or results of operations.

Our ability to use our net operating loss carryforwards and certain other tax attributes may be limited.

We have incurred substantial losses during our history and do not expect to become profitable in the near future and we may never achieve profitability. To the extent that we continue to generate taxable losses, unused losses will carry forward to offset future taxable income, if any, until such unused losses expire. Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, if a corporation undergoes an "ownership change," generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss carryforwards ("NOLs"), and other pre-change tax attributes (such as research tax credits) to offset its post-change income or taxes may be limited. The merger, our prior equity offerings and other changes in our stock ownership may have resulted in ownership changes. In addition, we may experience ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which are outside of our control. As a result, if we earn net taxable income, our ability to use our pre-change net operating loss carryforwards to offset U.S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liability to us. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.

Please refer to Item 3.02 contained in our Current Report on Form 8-K filed on October 2, 2017, our Current Report on Form 8-K filed on November 2, 2017, and our Current Report on Form 8-K filed on November 6, 2017 for the information required by Item 701 of Regulation S-K as to all equity securities that we issued during the period covered by this Quarterly Report on Form 10-Q that were not registered under the Securities Act.

Item 3. Defaults Upon Senior Securities.

None

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

None

Item 6. Exhibits.

Except as so indicated in Exhibit 32.1, the following exhibits are filed as part of, or incorporated by reference into, this Quarterly Report on Form 10-Q.

Exhibit Number	Exhibit Description
2.1(2)†	Agreement and Plan of Merger and Reorganization, dated September 26, 2017, by and among Max-1 Acquisition Corporation, Max-1 Acquisition Sub, a Delaware corporation and wholly-owned subsidiary of the Company, and Exicure OpCo, a Delaware corporation.
3.1(2)	Certificate of Merger relating to the merger of Max-1 Acquisition Sub with and into Exicure OpCo, filed with the Secretary of State of the State of Delaware on September 26, 2017.
3.2(2)	Certificate of Amendment to Certificate of Incorporation, filed with the Secretary of State of the State of Delaware on September 26, 2017.
3.3(2)	Form of Amended and Restated Certificate of Incorporation, to be filed with the Secretary of State of the State of Delaware.
3.4(2)	Amended and Restated Bylaws, as currently in effect.
4.1(2)	Form of Warrant to Purchase Shares of Common Stock issued to Placement Agent.

4.2(2)	Form of Registration Rights Agreement by and among the Company and the persons named therein.
10.1(2)+	2015 Equity Incentive Plan and forms of awards thereunder, assumed in the Merger.
10.2(2)+	2017 Equity Incentive Plan and forms of award agreements thereunder.
10.3(2)+	2017 Employee Stock Purchase Plan.
10.4(2)+	Form of Indemnification Agreement by and between the Company and each of its directors and executive officers.
10.5(2)	Form of Subscription Agreement by and between the Company and each investor in the initial closing of the Offering.
10.6(2)+	Form of Amended and Restated Board Member Service Agreement by and between Exicure OpCo and each of its non-executive directors.
10.7(2)+	Employment Agreement dated as of February 2, 2016 by and between Exicure OpCo and David A. Giljohann, Ph.D.
10.8(2)+	Amended and Restated Employment Agreement dated as of February 2, 2016 by and between Exicure OpCo and David S. Snyder.
10.9(2)+	Employment Offer Letter dated as of September 16, 2015 by and between Exicure OpCo and Ekambar Kandimalla, Ph.D.
10.10(2)+	First Amendment to Offer Letter dated as of January 8, 2016 by and between Exicure OpCo and Ekambar Kandimalla, Ph.D.
10.11(2)+	Consulting Agreement dated as of October 1, 2011 by and between AuraSense Therapeutics, LLC and Chad A. Mirkin, Ph.D.
10.12(2)	Lease Agreement dated as of February 13, 2012 by and between AuraSense Therapeutics, LLC and FC Skokie SPE, LLC.
10.13(2)	Letter dated as of March 12, 2012 regarding the Lease Agreement by and between AuraSense Therapeutics, LLC and FC Skokie SPE, LLC.
10.14(2)	First Amendment to Lease dated as of March 31, 2014 by and between AuraSense Therapeutics, LLC and FC Skokie PQ, LLC, as successor in interest to FC Skokie SPE, LLC.
10.15(2)	Second Amendment to Lease dated as of May 26, 2016 by and between Exicure OpCo and FC Skokie PQ, LLC, as successor in interest to FC Skokie SPE, LLC.
10.16(2)	Loan and Security Agreement dated as of February 17, 2016 by and between Exicure OpCo and Hercules.
10.17(2)	Amendment No. 1 to Loan and Security Agreement dated as of October 10, 2016 by and between Exicure OpCo and Hercules.
10.18(2)+	Form of Pre-Merger Indemnity Agreement.
10.19(1)	Form of Common Stock Purchase Agreement.
10.20(2)*	Restated License Agreement between Exicure OpCo and Northwestern University dated as of August 15, 2015.
10.21(2)*	License Agreement between Exicure OpCo and Northwestern University dated as of February 10, 2016 and effective as of May 27, 2014.
10.22(2)*	License Agreement between Exicure OpCo and Northwestern University dated as of June 17, 2016.
10.23(2)*	Amendment One to the Amended Restated License Agreement between Exicure OpCo and Northwestern University dated as of September 27, 2016.
10.24(2)*	Research Collaboration, Option and License Agreement between Exicure OpCo and Purdue Pharma L.P. dated as of December 2, 2016.
10.25(2)	Side Agreement to Northwestern Agreements by and among Exicure OpCo, Northwestern University and Purdue Pharma L.P. dated as of October 11, 2016.

10.26(3)	Form of Subscription Agreement by and between the Company and each investor in connection with the Subsequent Closing.
10.27(3)	Form of Purchaser Rights Letter to be delivered by the Company to each investor in the initial closing of the Offering.
31.1(4)	Rule 13a-14(a)/15d-14(a) Certification of Principal Executive Officer
31.2(4)	Rule 13a-14(a)/15d-14(a) Certification of Principal Financial Officer
32.1**	Section 1350 Certifications of Principal Executive Officer and Principal Financial Officer
101.INS(4)	XBRL Instance Document
101.SCH(4)	XBRL Taxonomy Extension Schema Document
101.CAL(4)	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF(4)	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB(4)	XBRL Taxonomy Extension Label Linkbase Document
101.PRE(4)	XBRL Taxonomy Extension Presentation Linkbase Document

† Annexes, schedules and/or exhibits have been omitted pursuant to Item 601(b)(2) of Regulation S-K. We hereby undertake to furnish supplementally a copy of any of the omitted schedules and exhibits to the SEC on a confidential basis upon request.

+ Indicates a management contract or compensatory plan.

* Portions of this exhibit have been omitted pursuant to a request for confidential treatment, and omitted portions have been filed separately with the SEC

** The certifications attached as Exhibit 32.1 that accompany this Quarterly Report on Form 10-Q are not deemed filed with the SEC and are not to be incorporated by reference into any filing of Exicure, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of such Form 10-Q), irrespective of any general incorporation language contained in such filing.

(1) Incorporated by reference to the indicated exhibit in Max-1 Acquisition Corporation's Current Report on Form 8-K filed on June 19, 2017.

(2) Incorporated by reference to the indicated exhibit in our Current Report on Form 8-K filed on October 2, 2017 and as amended by our Current Report on Form 8-K/A filed on November 7, 2017.

(3) Incorporated by reference to the indicated exhibit in our Current Report on Form 8-K filed on November 2, 2017.

(4) Filed herewith.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

Date: November 14, 2017

EXICURE, INC.

By: /s/ David S. Snyder

David S. Snyder

Chief Financial Officer

(Principal Financial Officer and Principal Accounting Officer)

Exhibit 31.1

CERTIFICATIONS

I, David A. Giljohann, Ph.D., certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Exicure, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act rules 13a-15(f) and 15d-15(f)) for the registrant and have:

a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 14, 2017

/s/ David A. Giljohann, Ph.D.

David A. Giljohann, Ph.D.

President and Chief Executive Officer

Exhibit 31.2

CERTIFICATIONS

I, David S. Snyder, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Exicure, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act rules 13a-15(f) and 15d-15(f)) for the registrant and have:

a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under my supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 14, 2017

/s/ David S. Snyder

David S. Snyder

Chief Financial Officer

Exhibit 32.1

SECTION 1350 CERTIFICATIONS*

Pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. § 1350), David A. Giljohann, Ph. D., President and Chief Executive Officer of Exicure, Inc. (the "Company"), and David S. Snyder, Chief Financial Officer of the Company, each hereby certifies that, to the best of his knowledge:

1. The Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2017, to which this Certification is attached as Exhibit 32.1 (the "Quarterly Report"), fully complies with the requirements of Section 13(a) or 15(d) of the Exchange Act; and

2. The information contained in the Quarterly Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: November 14, 2017

/s/ David A. Giljohann, Ph.D.	/s/ David S. Snyder
David A. Giljohann, Ph.D.	**David S. Snyder**
President and Chief Executive Officer	**Chief Financial Officer**

* This certification accompanies the Quarterly Report on Form 10-Q, to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of the Company under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Quarterly Report on Form 10-Q), irrespective of any general incorporation language contained in such filing.